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RED CROSS REPORT

人道公益系列报告

2022中国重症肌无力患者健康报告

2022 Health report of patients with myasthenia gravis in China

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创造人道和有尊严的生活 To Build A Life With Humanity And Dignity

北京爱力重症肌无力罕见病关爱中心

Aili Myasthenia Gravis Care Center

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中国红十字基金会
CHINESE RED CROSS FOUNDATION
CRCF

2022 中国重症肌无力患者健康报告

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信息贡献：

961 名患者

92 名家属

7 名医生

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2.演讲：《从〈2022 中国重症肌无力患者健康报告〉透视患者需求》；

3.演讲：《中国重症肌无力患者健康报告 2022》；以及

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序　一

总结过去，展望将来

“重症肌无力”是一种累及神经与肌肉的接头处突触后膜上乙酰胆碱受体的自身免疫性疾病，其临床特征是活动后加重、休息后减轻的随意肌无力。在我国，发病率约为7.4例/百万人/年，属罕见病。18～35岁的女性和35～55岁的男性为高发人群。目前我国约有重症肌无力患者约65万人。当病情严重时，患者可能因累及咀嚼、吞咽、呼吸，需要用呼吸机辅助呼吸，甚至引发“重症肌无力危象”而危及生命。

由于社会公众、部分医务人员及患者对该疾病缺乏足够认识，54.15%的患者曾因被误诊，36.68%的患者曾因发现及治疗延误而出现过重症肌无力危象，更有患者甚至因缺乏治疗信心而自杀。

《中国重症肌无力患者健康报告》调研了1200多位中国重症肌无力患者的生存现状，发现了重症肌无力患者在生活工作及治疗中的诸多问题。希望能得到社会各界的关注，提升医疗水平，减少误诊、漏诊。加强科普宣传工作，让社会大众认识重症肌无力，为患者的积极康复创造良好的社会环境，大幅提升患者的生活质量。

（以下据许老于2022年4月22日本次调研工作启动会上的发言整理：）

中国重症肌无力患者健康报告是一个系统的总结，从报告成果未来应用的角度也帮我们展望了未来，指了应该走的方向，今后我们应该按照这个方向去努力，共同把我们工作做得更好。

另外，我们也应该比较系统回顾一下以前，国内在研究方面，实验室啊，临床治疗啊做了一些什么？ 我觉得对于指导今后进一步的工作也很重要。

譬如说，上个世纪90年代初，我们跟一个部队医院合作，做激素应用的治疗效果，包括它的效果怎么样啊？ 副作用怎么样的？ 启动的时候会有什么样的反应？ 我们那个时候得到的一个印象是，如果用激素大量冲击是一个礼拜之内会加重的。 如果说超过了一个礼拜，一般是不会加重的。 这些结果对指导我们激素临床应用是有帮助的。

还有呢，我们以前也有过教训。 有一个患者是内蒙的医生他来了，他来了以后我们跟他说，激素也可以慢慢加，但小剂量往上加的话呢，也是有可能加重的。 他说我是医生嘛，我就回去慢慢加吧，有什么事我再跟你联系。 结果他小剂量5毫克加到50毫克的时候病情加重了，来了一个电话，说许大夫我不行了，我来不了，病情加重了，走路也走不了了。 这就告诉我们什么呢，加激素的问题，你给好多病人加激素都觉得没有什么加重的，实际上小剂量往上加了一个礼拜两个礼拜出院了，（病人）加重（没回来），跑别的医生那去（看）了，不在你这了，所以原来的医生（做）总

结的时候（这个病人）绝对是没有加重。

所以我就觉得好多东西，今后我们一定系统的把过去的经验和教训总结总结，来指导我们今后更好的工作，这点是很重要的。今天不是说这些具体的方法、问题的时候，主要是指出一个努力方向。总结过去，总结经验教训是为了更好地指导今后的工作和努力方向。我想这是非常重要的。

另外，关于确诊的延迟的问题。我想主要是这方面的认识不够，有两个层面。一个是病人的层面，普通人群一般老百姓。第二个是医护人员方面也不是很认识，病人去了医生也不知道。每年的国际国内的会都提到这个方面的重要性，所以呢，在普通人群跟医护人员两个层面上做了比较多的宣传的工作，这是非常重要的。

还有一个复发的问题。以前上个世纪 90 年代初的时候，我们跟一个部队医院共同做了一个协作者研究，用激素治疗以后到底会不会加重？做了这个（研究后）有一篇文章发表，也到北美的会议上做了一些报告。总的结论就是说，你要用激素要随访两年，就是用激素的开始的时候，如果用大剂量是 1000 毫克，甚至是 2000 毫克一天，那么用一个礼拜，几天就加重了，要么就是不加重，一个礼拜之内不加重后头就不加重了，这就是随访两年的结果，不是说看一个礼拜两个礼拜。还有呢，有的说小剂量就不加重。结果呢？我们看了有些人，五个毫克十毫克一点点的加不加重，加到了 50 毫克 60 毫克的时候加重了。所以，用小剂量不加重，这个依据是不够的。要得出这样的结论的话，要有更多的事

实证据，可能更好一些。

那么，最后一个生育的问题。我想这是对于女病人，大家注意的比较多，但是男病人有没有问题呢。病本身对于男病人的影响，还有用药对男病人的影响，会对生理带来什么不利影响没有?这方面缺乏事实根据。

我举个例子，疾病指南是有法律效应的。按指南办错了，没问题。如果你不按这个指南办，错了，要负法律责任了。比如，多发性硬化的指南说，（醋酸格拉替雷）对于多发硬化病人的怀孕没有什么不利的影响。如果说病人在治疗期间怀孕了，要改药就改成它，如果治疗期间还在用着还可以继续再用，他的结论就是（这个药）是安全的。但是去年美国的眼科杂志送了一篇文章来，叫我们审，我就审了一下。这个病人是多发性硬化病人，是用了（药）结果发生了畸形。我们建议他发表，为什么呢?尽管说这个孩子有畸形，不能说明他两者之间的因果关系，但这两个在时间上是有关系的。那么以前指南所说的，改成这个药是安全的。现在建议他发表这篇文章，就是引起大家对这个问题的今后的重视，而不是说他就是因果关系了。那这篇文章也是发表了。

对重症肌无力来说，这方面研究恐怕更缺乏。比如疫苗的问题，打了疫苗有没有好处?对病来说打疫苗的好处是能不能预防（新冠），坏处是（肌无力）能不能加重或复发。这个问题应该谁来回答，应该是打疫苗的人来回答。如果这个病人打了复发的副作用不大，预防他今后感染的作用很大，这样得失两方面的比较，才能得出一个结论。

我觉得呢，要有更多的事实，实际的材料，像这次的报告研究的内容，随访收集的材料，要有一个实事求是的态度。我经常对我的学生说，医生看得不是病，是病人，观察研究得越细致，就对病人越来越好。

那么总结过去，展望将来。回顾过去，我们取得了很多成绩，展望将来，一定会取得更好的成绩，但需要做更多的工作。我就说这些，更宝贵的时间留给后面的医生。

原卫生部北京医院主任医师
原北京爱力重症肌无力罕见病关爱中心名誉理事长
许贤豪

编者按：

本报告序言撰写期间，许教授因病与世长辞，我们悲恸无比。许老一生致力于神经免疫学科的建设，在重症肌无力领域留下了重要的研究与临床成果，并深受广大患者爱戴。晚年长期致力推动重症肌无力行业发展和公益事业进步。为表达我们最深切地怀念和感激之情，本报告保留了许老未完的序言以及于报告启动会中三段发言的内容，谨此表达追思，代表中国重症无力患者为许教授一生在重症肌无力临床诊疗和疾病研究所做付出的心血和取得贡献，致以深深谢意与崇高敬意！

序 二

携手推动 罕见病保障

2022年重症肌无力患者健康报告里面提供了大量的信息，应该说是全景式的描绘了重症肌无力患者的生活状态，健康状态，以及他们在救治过程中所经历的重要经历。

特别是这次的报告，重点还做了33名患者的个案的深度访谈，首次发布了中国重症肌无力患者的疾病旅程，非常真实的全景式的给我们展现了重症肌无力患者在治病救治，包括他们现在的一些健康状况，以及他们对社会的基本需求。

对于重症肌无力患者的治疗，特别是对他们的医疗保障工作，政府是高度重视的。 这几年我们生产和研发的治疗重症肌无力患者的药品，基本上全部都纳入了医保。 这样就不仅能够保证重症肌无力患者有药可治，而且还能够通过医疗保障制度之后发生的医疗费用，可以在他们的承受范围之内。 同时，在这个报告里我们可以看出，对于重症肌无力患者的治疗，这是一个决定性的原因，是解决重症肌无力患者健康问题的一个重要的手段。

但是我同时认为关注包括重症肌无力患者在内的罕见病，其他方面的生活需求也是非常重要的。 比如说：

第一、要给他们提供一个安全和方便的生活环境，从我们的公交车，从我们的马路建设等等这方面，都需要提供一个便于他们出行的生活环境。

第二、在就业上面要采取一些好的政策，使得这些患者能够公平的就业。因为就业是民生资本，如果没有就业的话，患者就没有收入，家庭就会陷入贫困，使他们的生活处于一种贫困状态。我们要公平的解决他们的就业问题。

第三、我们要逐步建立长期照护保险制度，因为我看有很多重症肌无力患者，他们是需要半护理和全护理的状况，那么这也会给家庭给个人带来很强的一些经济负担。

所以在这里也呼吁我们全社会要关注，包括重症肌无力在内的所有的罕见病患者，他们的一个生活状态和疾病治疗的状态。

这份报告我还有几点建议，第一个就是在有一些现状描述里边，有一些数据给了我们很好的能够展示重症肌无力患者比如说他们的就业状态，他们的教育状态等等，但是缺乏有一个和其他人群的比较研究的一些数据，所以就使得我们只能静态的看这些病人的一些相应的数据，但是没有比较就不能说明差距。

第二个对于发现的很多问题，我们需要做一些原因上的分析。在读这个报告的时候，我非常关注重症肌无力患者的药品纳入医保的情况，我看见的是所有的药品都纳入了医保，但是却有很多患者报告显示没有得到报销，这就要分析它的一些原因，到底是因为他们发生的费用不够医保的支付标准，还是因为他们都是看门诊，有些地方只是住院报销等等，所以建议做这些原因的分析，只有这些

原因的分析，我们才能够发现问题所在，才能够给政府相关部门提供一些政策上的建议。

还有一个问题，就是比如说还有不少的患者，他没有参加疾病医疗保险，到底是因为什么没有参加？ 是我们的政策的宣传力度不够？ 还是他们就没有钱缴纳这个疾病医疗保险费？ 还是有什么其他的原因呢？ 所以我觉得相应的结果的发现，最好能够做原因的分析。

总体来说，这是一份难得的报告，我也希望这个报告能够引起全社会的关注，能够提供给相关部门，让他们在制定政策中参考，也能在学术界为相关学术方面能够引用这些报告的数据。

让我们携手起来，能够共同推动，包括重症肌无力在内的罕见病的保障工作。 我们也要积极推动罕见病的立法，使罕见病病人，包括重症肌无力患者能够生活在公正公平的社会环境之下！

国家医疗保障局医药服务管理司原司长（正司级）
熊先军

前　　言

2023 年 2 月，注定是将要留下记忆的时间。 三年的疫情防控放开，生活逐渐回到它本来的样子，而这也是北京爱力重症肌无力罕见病关爱中心创办刚好十周年。

十年间，爱力完成了 4 次对中国重症肌无力患者的大规模调研工作，发布的各类报告在公益领域和重症肌无力科研领域都产生了重要的推动力。 报告内容通过媒体、自媒体、印刷品、电子刊物等形式，在各种渠道传播、传递，一对一发放纸质报告 4000 册，上百次外部分享活动中都有报告相关的内容分享。 报告有效问卷数分别是 2014 年 614 份，2018 年 1896 份，2019 年 2397 份，本次调研有效问卷 1020 份并增加了 33 例患者与家属个案访谈和 7 例医生访谈，访谈时长 2—5 小时，这 5967 份样本量折射着调研工作的变化、社会环境的变化，不变的是患者们对生活美好的希望，对疾病治愈的渴望。

千里之行，积于跬步。

成立之初，爱力在各类场所宣传重症肌无力患者的情况时，每说到患者艰辛的疾病旅程、未满足的药物需求、保障的匮乏、生存状况质量之低下等现状，希望能引起媒体、政府、药企等各方关注时，除了病友自强不息的生命故事令人唏嘘，常被反问的是：有数

据吗？ 人群是多大？ 危象发生的概率是多少？ 某种困境的覆盖面是多大？ 人年平均治疗费用是多少？ 诸如此类。 我和另一位创始人海连大哥总是很难回应，这也让我们下决心要进行患者的生存情况调研。

2014 年《中国重症肌无力患者生存报告》首次发布，年龄跨度从最小的 4 岁幼儿患者至 80 岁的老年患者，过去仅靠印象判断的问题被数据清晰呈现。 令人兴奋的是，许多结果与服务中的主观观察相稳合，得到了印证。 以报告为契机，爱力在当年联合社会各届发起了 6 · 15 重症肌无力关爱日，首次向公众呈现了社会视角下的中国重症肌无力患者生存状况，有病友拿着报告找到当地医保部门，成功呼吁将重症肌无力纳入门诊报销。

此后，2018 年发布了《2018 重症肌无力患者生存报告——中美比对视角》，其内容令人震撼，中美患者生存质量的较大差距反映出中国患者更加艰难的现状，药物使用的差别反映出患者认知、医生治疗水平的明显区别，报告背后，是患者依旧艰难的生活处境与巨大的未满足需求。 2018 年的调研，还首次关注了并发症对重症肌无力患者的影响以及情绪心理、生活质量评价、性别差异等。

也在 2018 年，国家多部委联合发布《中国罕见病名录》，重症肌无力位列其中，以此契机，患者的生存状况、未满足需求和爱力的工作都得到了空前关注，而国际领域新药研发的声音也陆续传来。

这是爱力成立最初五年的经历，无论科普倡导、患者援助、疾病教育，还情绪疏缓、社群赋能、社会融合等各种围绕改变患者生

存状况的服务工作和倡导发声，都因最初未被看见的微小努力而有了新的曙光，让我们感受到了不一样的意义——它其实可以被看见，其实改变正在发生。

此后的五年，各种政策出台和媒体声音对罕见病形成了推力，重症肌无力是其中受益的一份子，也是推动力的一部分。

从单次调研看，这是一件脚踏实地、深耕细作的数据信息工作；从时间跨度看，爱力的系列调研背后却是一个宏大叙事。

十年后的今天，我们从报告呈现看见，患者的确诊时间在显著缩短，危象发生率、呼吸机使用率有所下降，患者可选择的药物在增加，认知在提升，症状在改善，困境点从纯粹的生存问题转向需要更好更安全的药物和疾病管理问题，保障问题从最初的地方门诊覆盖逐渐转向新药报销多方支付。

所以这次调研过程，关注点从患者的生存状况聚焦到健康层面，通过 33 例深访个案，首次形成了“中国重症肌无力病患旅程”，辅以问卷数据，对患者旅程各阶段遇到的问题，如就诊和疾病管理、依从性、患者和家属视角、复发和病情变化过程进行了真实呈现。

重症肌无力对患者是一条曲折且起伏的长路。

我们希望以时间的维度看宏观，从个体的不同看人生，深层次地探询患者在疾病困扰下的健康影响，群体和个体都同样重要。我们希望为各方提供在后疫情时代下的思考，帮助政府部门、医院、医学专家、药企、服务提供者在新的社会环境中，更准确把握重症肌无力患者面临的健康痛点和解决困难点，这或许是成为疾病

被彻底治愈、问题被彻底解决的蝴蝶翅膀。

报告的发布，承载着许多人的心血，在此，代表爱力和病友们深深感恩各届的努力和支持。

遗憾的是，报告撰写期间，十年来一直在支持爱力各类工作的，本届调研工作的医学顾问、爱力关爱中心名誉理事长、神经免疫学泰斗许贤豪教授与世长辞，愿报告的发布告慰他毕生推动重症肌无力临床与科研工作的拳拳之心、殷殷之情。

本次调研由中国红十字基金会大力支持，作为《红皮书》报告系列中目前唯一的罕见病病种报告，期待被更多相关方看见和重视。

爱力关爱中心理事、香港中文大学深圳研究院副研究员、罕见病真实世界数据研究实验室负责人董咚教授带领团队进行了非常辛苦的调研分析，发现了许多新的关注点，她自 2015 年就关注重症肌无力社会研究，主持策划了各类重症肌无力调研工作，发布了许多相关研究成果。

同时，感谢医学顾问湘雅医院神经内科主任医师杨欢教授，调研工作阶段正值疫情影响，医生为整个社会担负起了责任，繁忙中她花许多精力支持本调研的开展。 还要感谢参加本次调研访问的七位中西医重症肌无力专家在休息时间接受访谈，贡献了医生视角下的宝贵信息，出于访谈的信息保护，这里不再公开致谢。

还要感谢 1053 名患者和家属贡献信息，感谢捐助方杨森中国的重要支持，感谢研究团队每位成员、爱力团队专兼职伙伴、志愿者和患者关怀员的投入与奉献！

也特别感谢长期为爱力的调研工作给予帮助的华山医院赵重波教授、宣武医院李海峰教授、北京医院殷剑教授。

重症肌无力就像一片雪花，千人千面没有一片相同，也如同雪花可以被温暖融化。数据后是每一个独特的生命，都值得被看见、尊重，人人拥有平等的生存权、医疗权，共享健康和生命的幸福感。

愿不再听到悲伤的故事，让无力者有力，让有力者前行，让世界被爱与温暖沐浴！

北京爱力重肌无力罕见病关爱中心
创始人/主任：清昭
于二零二三年二月

清昭：爱力关爱中心发起人、主任，2002 年确诊重症肌无力，2006 年开始为病友群体服务，2013 和病友创办爱力关爱中心。秉承着助人自助，自助助人的理念带领越来越多的病友一直走在公益道路上。

目　　录

第一部分
研究缘起与调研简介

1. 调研概况

1）疾病背景与调研目的

重症肌无力（myasthenia gravis，MG）是由自身抗体介导的获得性神经-肌肉接头（neuromuscular junction，NMJ）传递障碍的自身免疫性疾病。

其症状包括不同程度的肌肉无力，最常影响眼部、脸部及吞咽相关的肌肉，可能会造成复视、眼睑下垂、说话困难及行走困难等症状。乙酰胆碱受体（acetylcholine receptor，AChR）抗体是最常见的致病性抗体；此外，针对突触后膜其他组分，包括肌肉特异性受体酪氨酸激酶（muscle-specific receptor tyrosine kinase，MuSK）、低密度脂蛋白受体相关蛋白 4（low-density lipoprotein receptor-related protein4，LRP4）及兰尼碱受体（RyR）等抗体陆续被发现参与 MG 发病，这些抗体可干扰 AChR 聚集、影响 AChR 功能及 NMJ 信号传递。

MG 全球患病率为（150～250）/百万，预估年发病率为（4～10）/百万。我国 MG 发病率约为 0.68/10 万，女性发病率略高；住院死亡率为 14.69‰，主要死亡原因包括呼吸衰竭、肺部感染等。各个年龄阶段均可发病，30 岁和 50 岁左右呈现发病双峰，中国儿童及青少年 MG（juvenile myasthenia gravis，JMG）患病高达 50%，构成第 3 个发病高峰。最新流行病学调查显示，我国 70～74 岁年龄组为高发人群。①

目前，重症肌无力的治疗以胆碱酯酶抑制剂、糖皮质激素、免疫抑制剂、静脉注射免疫球蛋白（intravenous immunoglobulins，IVIG）、血浆置换（plasma exchange，PE）以及胸腺切除为主，另外医学界在单抗类药物和生物靶向药方面也取得重要进展。本研究面向重症肌无力患者的日常生活，旨在从患者的病患旅程、医疗负担、社会支持与未满足需求等方面了解其生存现状，以期能推进公众和政府对重症肌无力的了解，改善重症肌无力患者的生活质量。

2）研究设计

本研究采用序列混合研究方法（sequential mixed methods），结合以深度访谈为主的定性研究与以横断面调查为主的定量研究，探索中国重症肌无力患者的生存现状。

本次调研的定性研究于 2022 年 1 月正式启动，于 2022 年 5 月截止。2022 年 1 月，开始招募患者访谈对象，经 70 余位患者及家属报名，从中挑选符合入组标准的患者及家属。访谈方法为半结构式深度访谈。每一例患者或家属访谈时长为 3-5 个小时。患者访谈提纲主要包括：基本情况、病患旅程（病发、确诊、就医、治疗、疾病日常管理等）、心理及生活支持、其他（例如经济负担、就业等）。4 月，开始医生访谈，研究团队总共访谈了 7 位医生，他们分别来自神经内科、胸外科、中医科，其中部分医生是患者访谈过程中发现与患者关系比较紧密的，由较多患者提起的医生。针对患者访谈中的病患旅程和就医体验，我们根据访谈医生的科室，制定了医生访谈提纲。每一位医生访谈时长约 1-2 个小时，访谈主要内容为：医生基本资料、与重症肌无力相关的诊疗经验、不同科室对诊断治疗与康复的理解、其

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① 中国免疫学会神经免疫分会. 中国重症肌无力诊断和治疗指南（2020 版）[J]. 中国神经免疫学和神经病学杂志，2021，28（1）：1-12.

他（生育、难治性、医患沟通、中医、心理、国外经验等）。截止2022年5月14日，总共完成患者或家属访谈33例，其中患者26人，家属7人，其中家属访谈里有3位是受访病友的家属，另外4位是单独访谈的家属，所以病患旅程信息为30位。此外该研究还完成医生访谈7例，其中神经内科医生3人，胸外科2人，中医2人。

本次调研的定量研究于2022年6月6日正式启动，于2022年7月13日正式截止。通过基于网络的全国性问卷调查，共收集有效问卷1020份。在有效问卷中，由患者自行填写的共计935份，由患者家属或其他主要照顾者代填的共计85份。问卷收集了患者的社会人口学信息、生活方式、病情特征、就诊经历、治疗情况、医疗支出、生活质量等资料，同时也收集了主要照顾者在社会支持与工作效率及活动能力方面的信息。

3）研究伦理

本研究已通过香港中文大学调查和行为研究伦理委员会的伦理审查（审查编号：SBRE-21-0260）。所有参与深度访谈和问卷调查的患者/家属和医生均经过详细的知情同意过程，对于访谈和问卷调查的意义、风险和收益等都给予完全的告知，并在获得同意之后才正式开始访谈与问卷填写。所有访谈资料全部经过转录，报告中所有患者已全部匿名。

4）数据分析

定性研究的数据分析分两阶段迭代进行。在第一阶段，两位研究员共同对十个访谈转录文本进行手动编码，通过对比文本与文本之间相似或相对的部分以确定重复出现的主题。第一阶段手动编码完成后，两位访谈员得以确认重症肌无力患者病患旅程的七个主要阶段：发病——确诊——治疗——复发与危象——病友组织支持——康复——当前病情及其他需求。同时，两位访谈员也发现每个主要阶段下可能浮现的子主题。在数据分析第二阶段，两位访谈员分别用质性分析软件Dedoose对所有访谈文本进行编

码分析，不断更新完善子主题的分类，并在编码完成后与研究团队交流讨论分析成果。

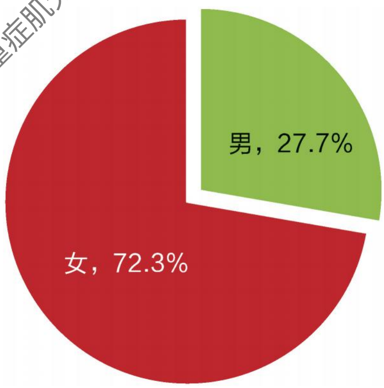
定量研究中，对不同变量进行统计描述，分类变量（如性别、年龄、户口等）采用频数（构成比）进行描述，连续变量（如收入、医疗支出、生活质量量表评分等）根据情况使用平均数、中位数、最小值及最大值进行描述。 所有统计分析使用统计软件 Stata 16.0 完成。

第二部分
重症肌无力患者生存现状调查

2. 人口学信息

1）性别

在 1020 名受访者中，男性患者共有 283 人，占比 27.7%；女性患者有 737 人，占比 72.3%。

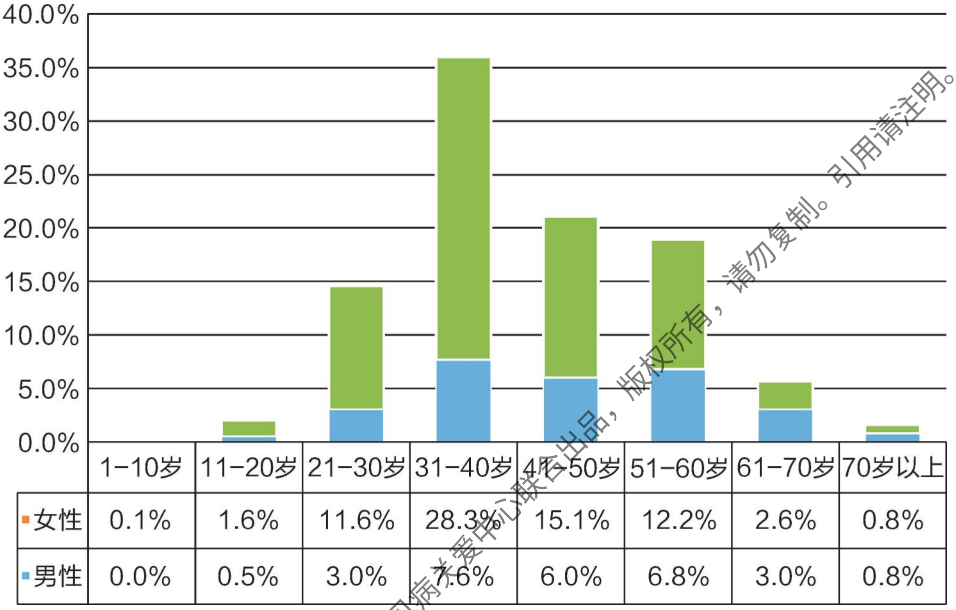


2）年龄

受访者中 31-40 岁年龄段的人数最多，共 367 人，占比 35.9%；其次是 41-50 岁的年龄段，共有 215 名患者，占比 21.1%；1-10 岁年龄段的患者数量

最少，共 1 人，占比 0.1%。 患者平均年龄为 41.7 岁，中位数年龄为 39 岁。

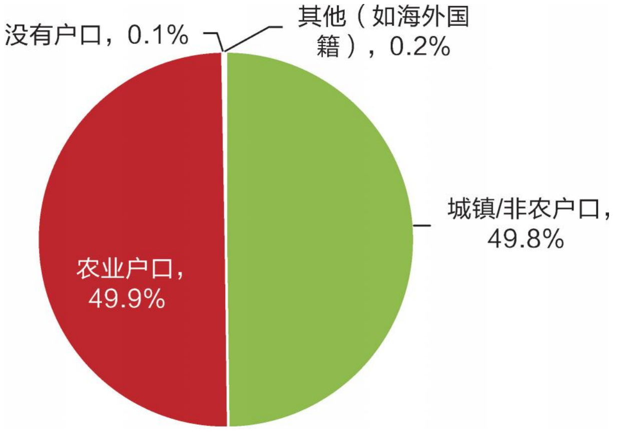
从男女比例来看，1-10 岁、61-70 岁、70 岁以上的年龄组男女比例相当，其他年龄组女性比例高于男性比例。



	1-10岁	11-20岁	21-30岁	31-40岁	41-50岁	51-60岁	61-70岁	70岁以上
🟧女性	0.1%	1.6%	11.6%	28.3%	15.1%	12.2%	2.6%	0.8%
🟦男性	0.0%	0.5%	3.0%	7.6%	6.0%	6.8%	3.0%	0.8%

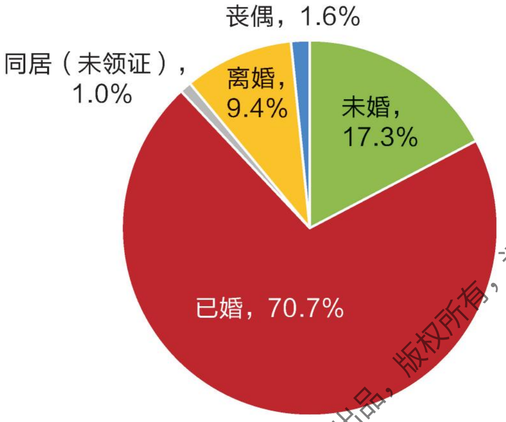
3）户口

受访者中农业户口共 509 人，占比 49.9%；城镇/非农户口共 508 人，占比 49.8%；有 2 人为海外国籍，占比 0.2%；1 人没有户口，占比 0.1%。



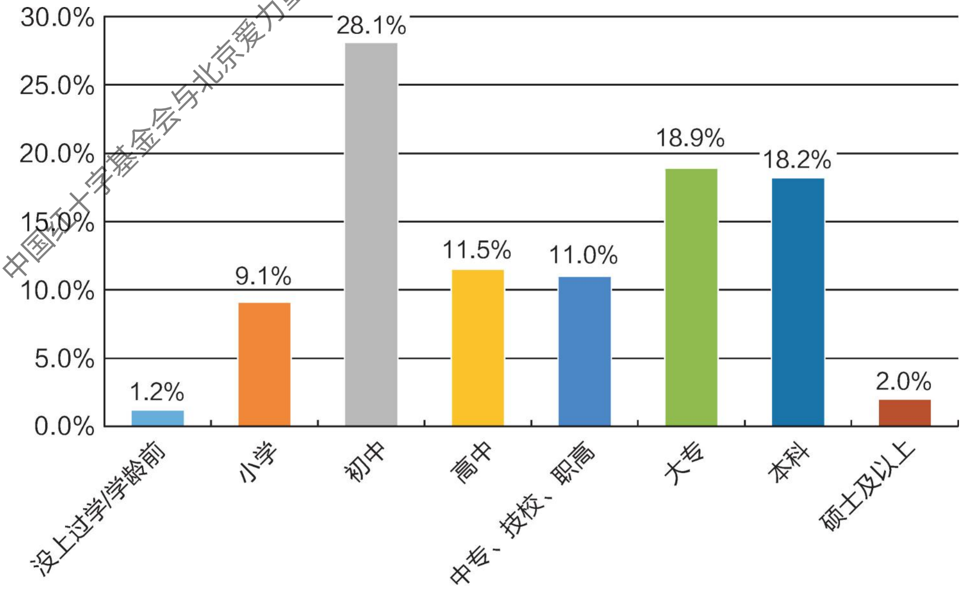
4）婚姻状况

成年受访者中已婚的人数最多，共 714 人，占比 70.7%；未婚共 175 人，占比 17.3%；同居的人数最少，共 10 人，占比 1.0%。



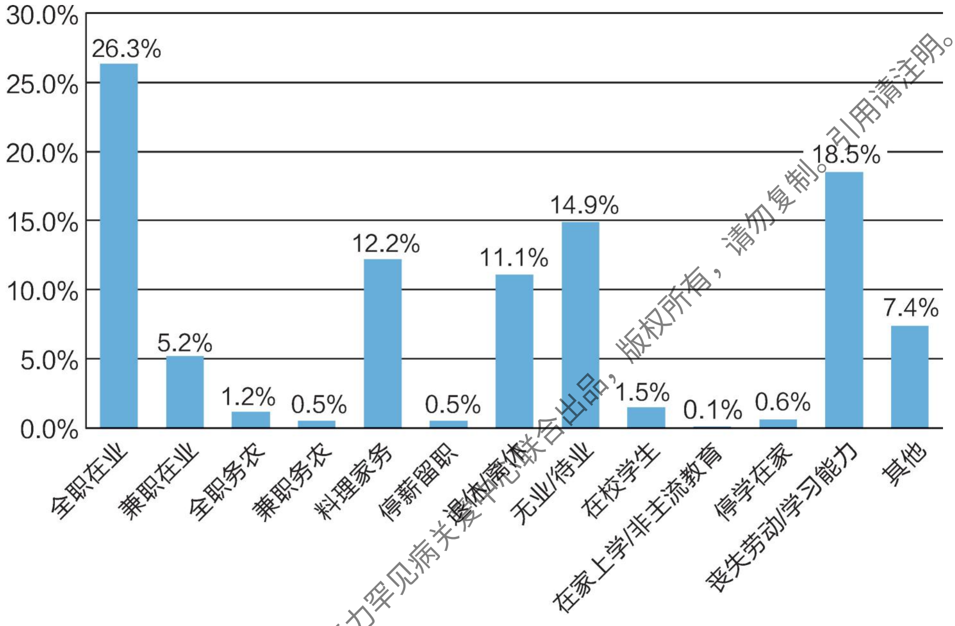
5）教育水平

受访者中，初中学历人数最多，共 287 人，占比 28.1%；其次是大专学历，共 193 人，占比 18.9%；没上过学/学龄前教育水平的人数最少，共 12 人，占比 1.2%。 整体上，接受过高等教育的人数比例为 39.1%。

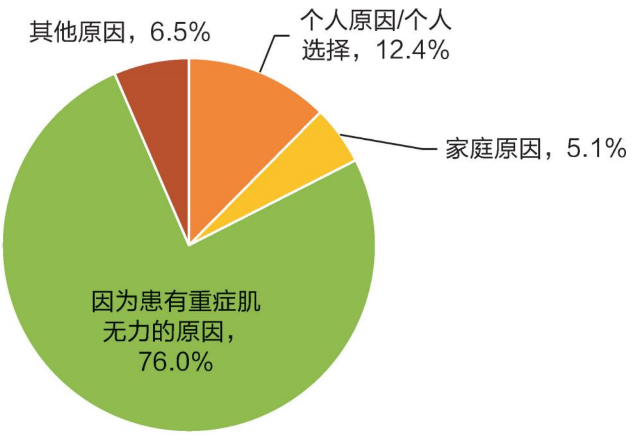


6）就业状况

在 1011 名成年受访者中，全职在业的人数最多，共 266 人，占比 26.3%；其次是丧失劳动/学习能力，共 187 人，占比 18.5%。



1011 名成年受访者中，未全职在业/在读/务农的人数共 612 人，占比 60.5%。 该人群中，造成目前就业/学业状况最常见的原因为重症肌无力，共 465 人，占比 76.0%；其次为个人原因/个人选择，共 76 人，占比 12.4%。



7）保险/保障状况

受访者中，享有城镇职工基本医疗保险的人数最多，共 371 人，占比 36.4%；其次新型农村合作医疗，共 149 人，占比 14.6%；共有 118 人没有任何健康医疗保险，占比 11.6%。

现有健康医疗保险	人数	%
城镇职工基本医疗保险	371	36.4%
新型农村合作医疗	293	28.7%
城镇居民基本医疗保险	149	14.6%
公费医疗	80	7.8%
补充医疗保险	66	6.5%
城乡居民合作医疗	64	6.3%
商业健康保险	54	5.3%
其他	13	1.3%
以上都没有	118	11.6%

共有 11.2% 受访者目前接受医疗慈善救助项目，其中 42 人获得民间罕见病救助资金，占比 4.1%；42 人为政府的医疗救助对象，占比 4.1%。

现有医疗慈善救助项目	人数	%
民间罕见病救助资金	42	4.1%
政府的医疗救助对象	42	4.1%
通过互联网平台进行众筹、募捐等	22	2.2%
其他	16	1.6%
以上都没有	906	88.8%

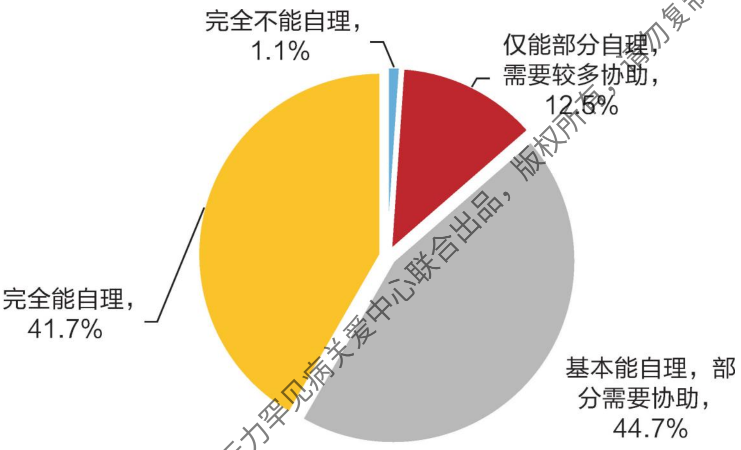
共有64.8%受访者目前享受社会保障/保险，其中享有养老保险的人最多，共350人，占比34.3%；其次为最低生活保障，共169人，占比16.6%；108人享有残疾人相关保障，占比10.6%。

现有保障/保险	人数	%
养老保险	350	34.3%
最低生活保障	169	16.6%
残疾人相关保障	108	10.6%
其他社会保障	34	3.3%
没有以上任何保障/保险	394	38.6%

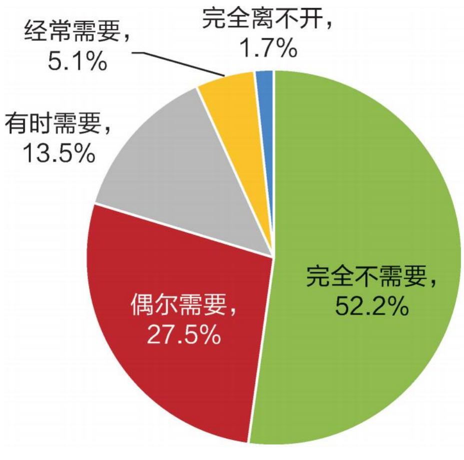
3. 身体功能与共患病

1）自理能力

受访者中，基本能自理的患者人数最多，共 456 人，占比 44.7%；其次是完全能自理的患者，有 425 人，占比 41.7%；需要较多协助的患者共 128 人，占比 12.5%。 完全不能自理的患者最少，仅有 11 人，占比 1.1%。

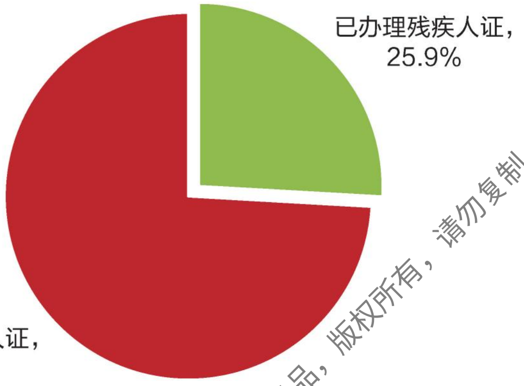


需要在日常生活中使用辅助器具的患者共 488 人，占比 47.8%；偶尔需要辅助器具的患者人数最多，共 281 人，占比 27.5%；完全离不开辅助器具的患者人数最少，共 17 人，占比 1.7%。

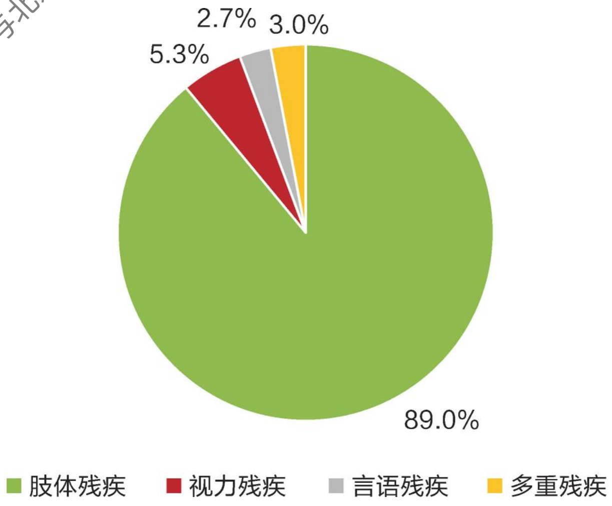


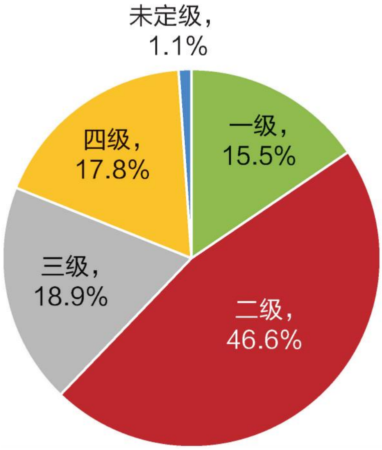
2）残疾证办理情况

在受访患者中，有264人办理了残疾人证，占比25.9%；756人目前尚未办理残疾人证，占比74.1%。

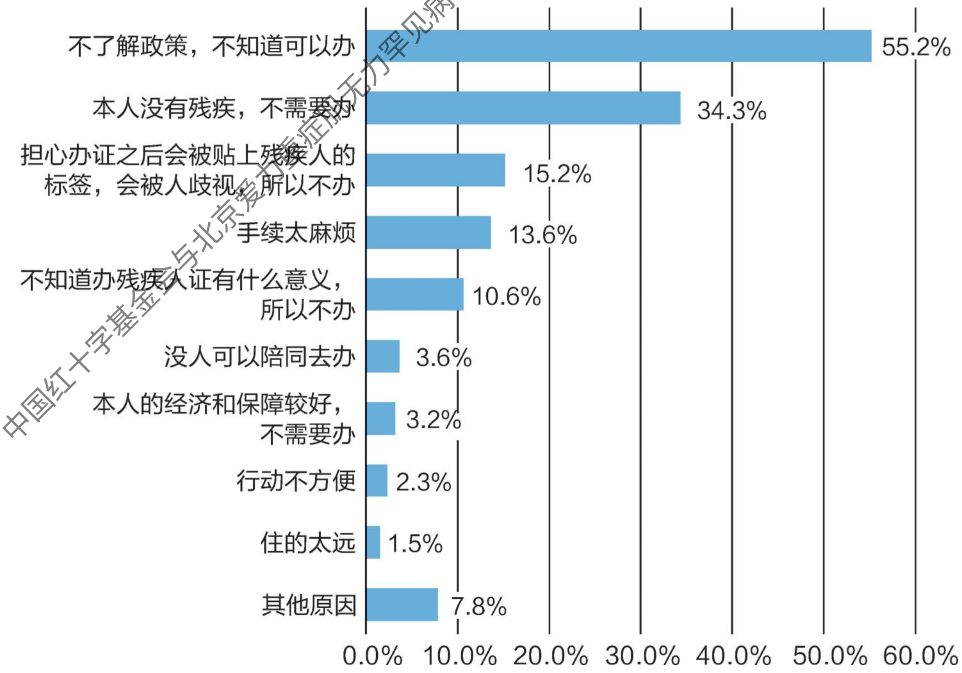


在已办理残疾人证的264名患者中，办理肢体残疾类别残疾证的人数最多，共235人，占比89.0%；其次为视力残疾，共14人，占比5.3%；残疾严重程度比较高的一、二级残疾占大多数，共164人，其中一级有41人，占比15.5%，二级有123人，占比46.6%；其次为三级残疾，共50人，占比18.9%。



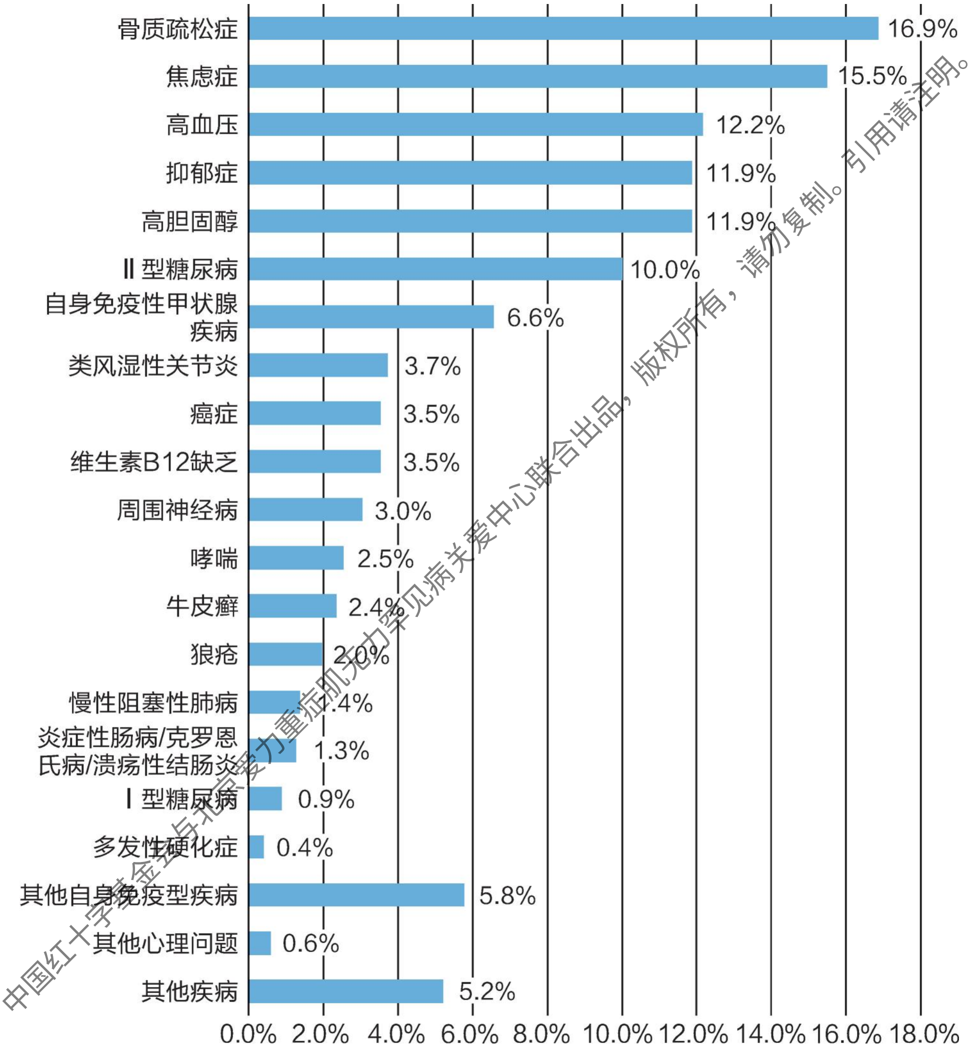


在 756 名目前尚未办理残疾人证的患者中，因为不了解政策，不知道怎么办儿没有办理的人数最多，共 417 人，占比 55.2%；其次为因为没有残疾不需办理的患者，共 259 人，占比 34.3%；因为住的太远而没办理的患者人数最少，11 人，占比 1.5%。



3）共患病

受访患者中，重症肌无力同时患有骨质疏松症的患者最多，共 172 人，占比 16.9%；其次为同时患有焦虑症的患者，共 158 人，占比 15.5%。



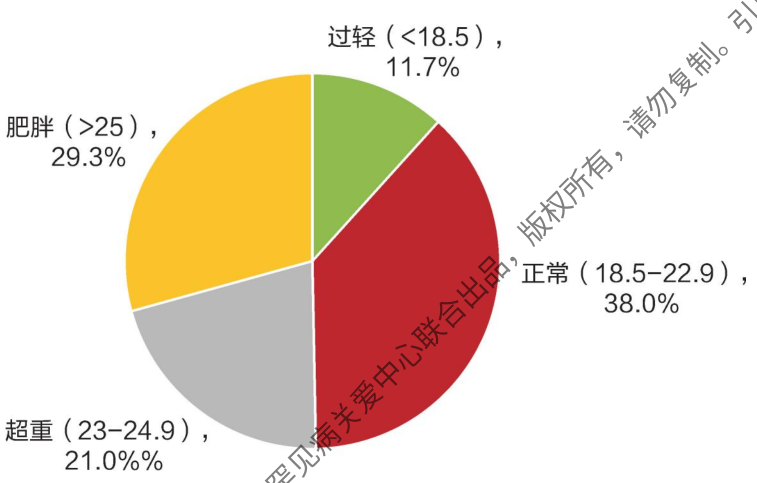
4. 生活方式

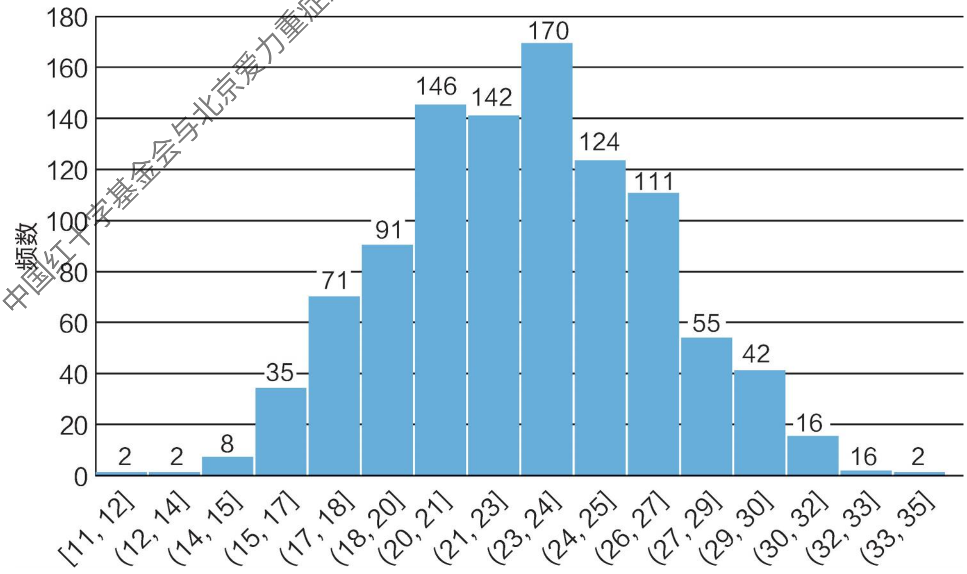
1）BMI

身体质量指数（BMI）是国际上常用的衡量人体胖瘦程度以及是否健康的

一个标准，计算公式为：BMI＝体重÷身高 2。

根据 WHO 对亚太地区 BMI 的分类，1011 名成年患者中，BMI 在正常范围内（BMI 18.5-22.9）之间的患者最多，共 385 人，占比 38.1%；肥胖（BMI＞25）的患者有 299 人，占比 29.3%；超重（BMI 23-24.9）的患者有 214 人，占比 21.0%；体重正常（BMI 18.5-22.9）的患者有 388 人，占比 38.0%；体重过轻（BMI＜18.5）的患者人数最少，有 119 人，占比 11.7%。



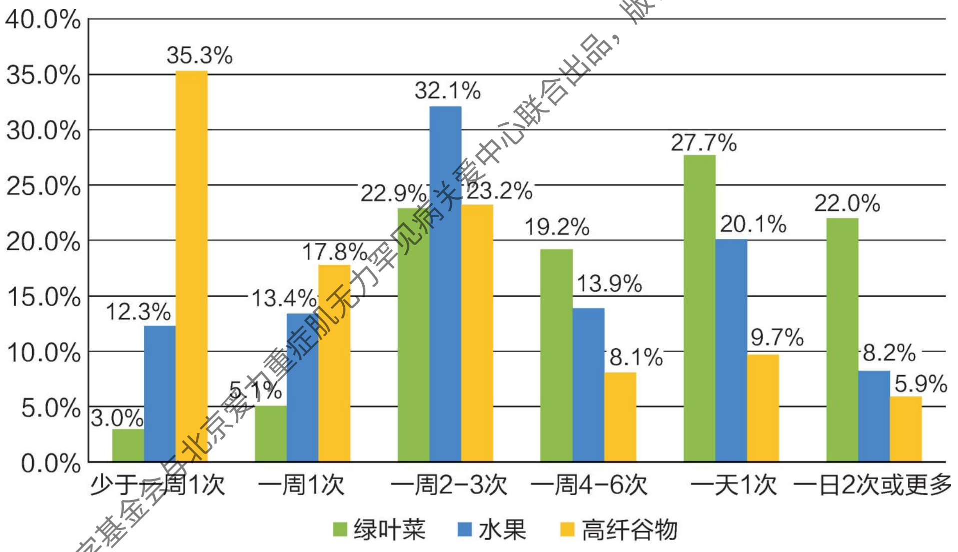


2）饮食

对于绿叶菜摄入，频率在每天 1 次的患者数最多，共 283 人，占比 27.7%；其次为一周 2-3 次的患者，共 234 人，占比 22.9%；31 名患者的频率少于每周一次，占比 3.0%。

对于水果摄入，频率在每周 2-3 次的患者数最多，共 327 人，占比 32.1%；其次为每天 1 次的患者，共 205 人，占比 20.1%；125 名患者的频率少于每周一次，占比 12.3%。

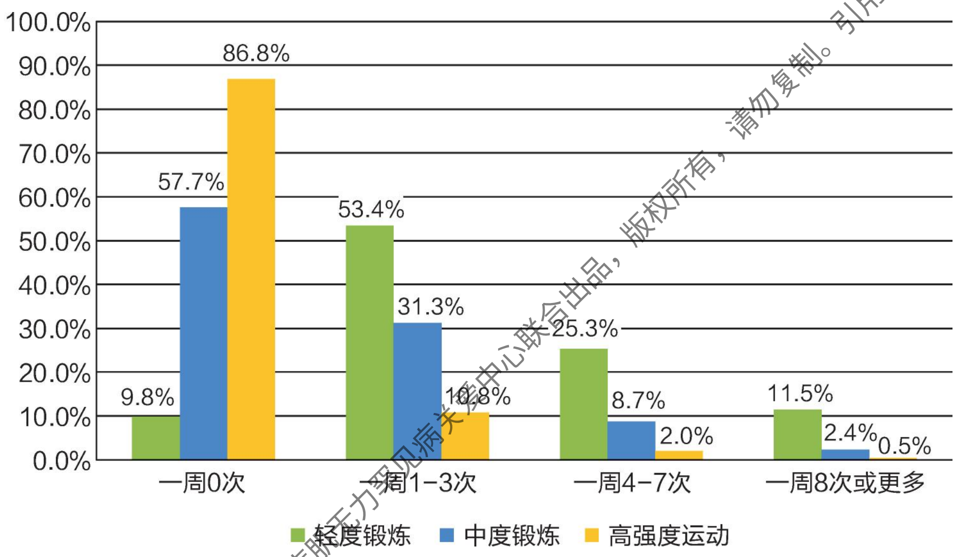
对于高纤谷物摄入，频率少于每周 1 次的患者数最多，共 360 人，占比 35.3%；其次为一周 2-3 次的患者，共 237 人，占比 23.2%。



3）运动

日常生活的中的锻炼有很多种不同的形式。 一般来说，轻度锻炼包括轻度的家务（如清扫、除尘），散步，逛商场等；中度的日常锻炼包括快走，骑单车，做园艺，跳舞打太极等；高强度锻炼包括跑步，有氧运动，耕种，足球篮球等剧烈球类运动等。

此次调研中发现，绝大部分 MG 患者不会进行高强度锻炼，而每周进行 1-3 次高强度锻炼的患者仅 10.8%， 共 110 人。 每周进行轻度锻炼的人最多，占比 90.2%， 其中轻度锻炼频率在每周 1-3 次的患者人数最多，共 545 人，占比 53.4%。 大约有 42.4% 的患者每周进行中等强度锻炼，其中每周进行 1-3 次中等强度锻炼的患者占 31.1%，共 317 人。



4）饮酒

所有受访患者中，饮用啤酒、葡萄酒、烈酒的患者比例分别为 5.4%、1.6%、2.6%。 4.4% 的患者饮用 1-5 单位啤酒/周，1.5% 的患者饮用 1-5 单位葡萄酒/周，2.1% 的患者饮用 1-5 单位烈酒/周。①

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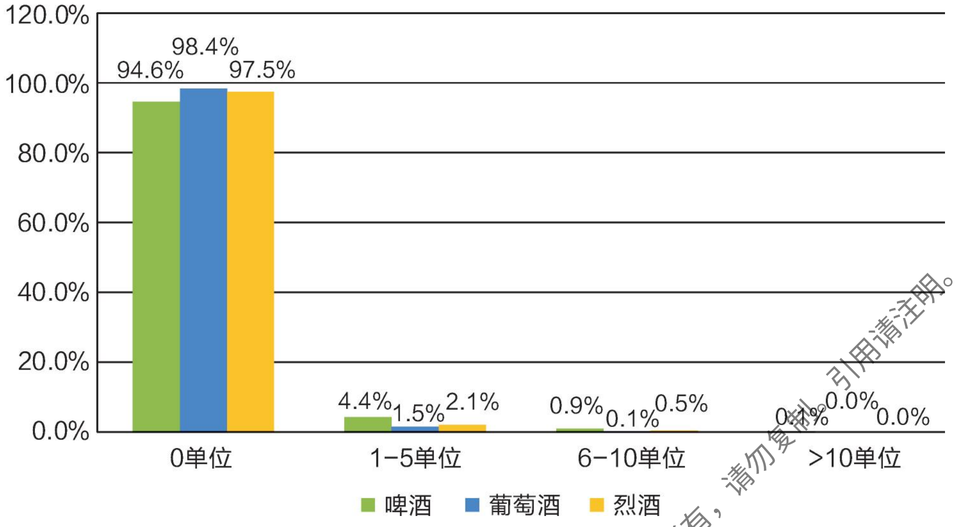
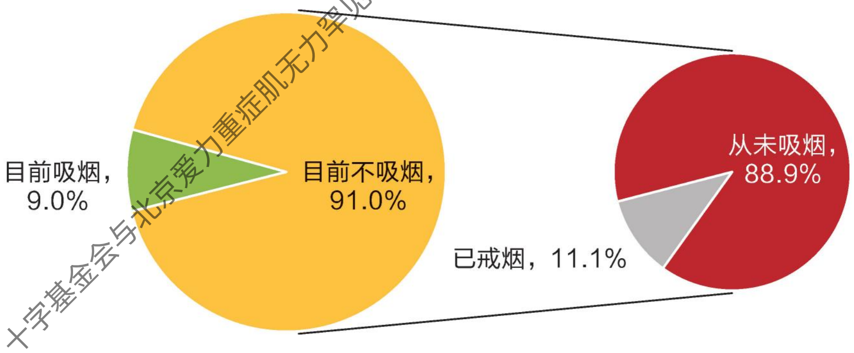
① 注：单位啤酒：约 284～340 毫升，330 毫升小易拉罐装啤酒一罐
位葡萄酒：约 85～142 毫升，125 毫升/1 高脚杯的红酒一杯
单位烈酒：约 28～43 毫升，白酒半两/至一两，例如二两装小二锅头半瓶



5）吸烟

所有受访患者中，92 人目前吸烟，占比 9.0%。 在 928 目前不吸烟的患者中，825 人从未吸烟，占比 88.9%；103 人已戒烟，占比 11.1%。



5. 诊断与检查

1）MGFA 分型

《中国重症肌无力诊断和治疗指南（2020 版）》中提出，关于 MG 患者的

分型，建议使用由美国神经病学学会与美国重症肌无力基金会（MGFA，Myasthenia Gravis Foundation of America）共同提出的 MGFA 分型来进行标识和区分。 MGFA 分型旨在对 MG 患者的疾病严重程度进行量化评估，但是并不会用于疗效和预后评价。

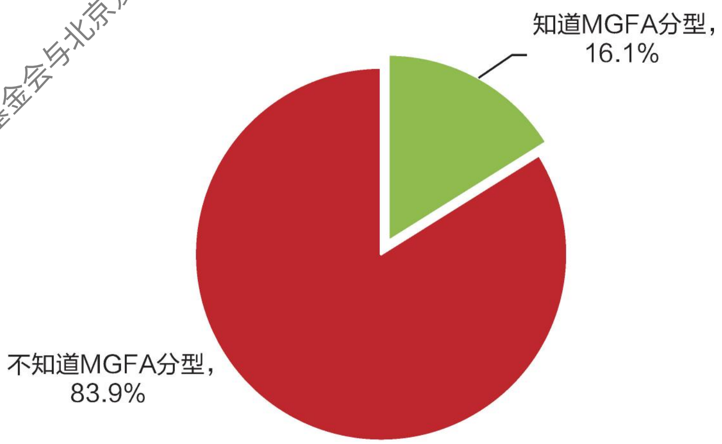
MGFA 分型具体如下：

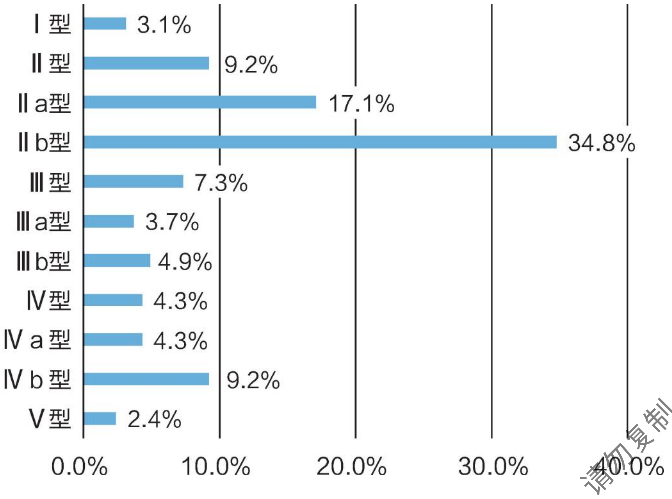
分型	临床表现
Ⅰ型	眼肌无力，可伴闭眼无力，其他肌群肌力正常
Ⅱ型	除眼肌外的其他肌群轻度无力，可伴眼肌无力
Ⅱa 型	主要累及四肢肌或（和）躯干肌，可有较轻的咽喉肌受累
Ⅱb 型	主要累及咽喉肌或（和）呼吸肌，可有轻度或相同的四肢肌或（和）躯干肌受累
Ⅲ型	除眼肌外的其他肌群中度无力，可伴有任何程度的眼肌无力
Ⅲa 型	主要累及四肢肌或（和）躯干肌，可有较轻的咽喉肌受累
Ⅲb 型	主要累及咽喉肌或（和）呼吸肌，可有轻度或相同的四肢肌或（和）躯干肌受累
Ⅳ型	除眼肌外的其他肌群重度无力，可伴有任何程度的眼肌无力

分型	临床表现
Ⅳ a 型	主要累及四肢肌或（和）躯干肌受累，可有较轻的咽喉肌受累
Ⅳ b 型	主要累及咽喉肌或（和）呼吸肌，可有轻度或相同的四肢肌或（和）躯干肌受累
V 型	气管插管，伴或不伴机械通气（除外术后常规使用）；仅鼻饲而不进行气管插管的病例为Ⅳ b 型

在受访患者中，164 人知道自己的 MGFA 临床分型，占比 16.1%；856 人不知道自己的 MG 临床分型，占比 83.9%。

知道自己 MGFA 分型的 164 名患者中，有部分患者不清楚自己具体属于哪一种亚型，因此只能回答所属的大类，例如 II 型、III 型或者 IV 型。 在知道亚型的患者中，以 IIb 型的患者人数最多，共 57 人，占 34.8%；其次为 IIa 型，共 28 人，占比 17.1%。 所有知道分型的患者中，以 V 型的患者人数最少，共 4 人，占 2.4%。

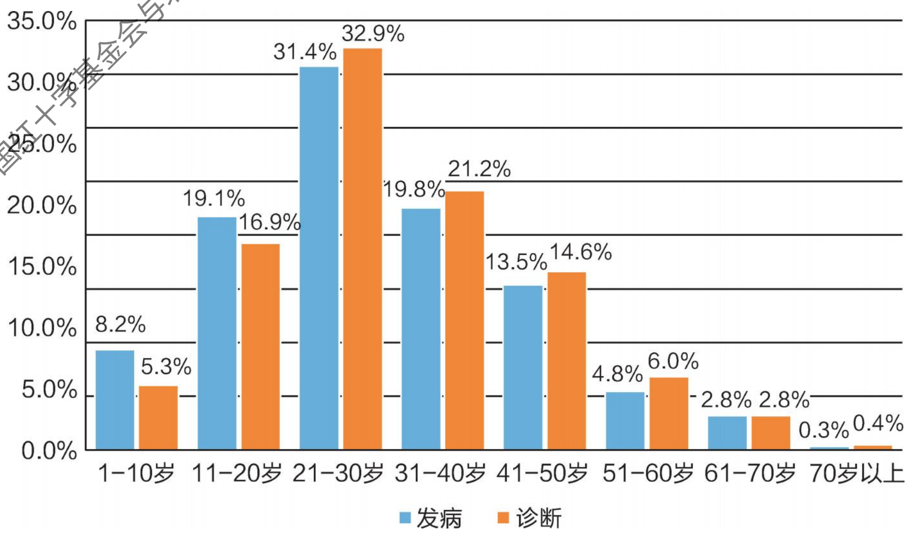




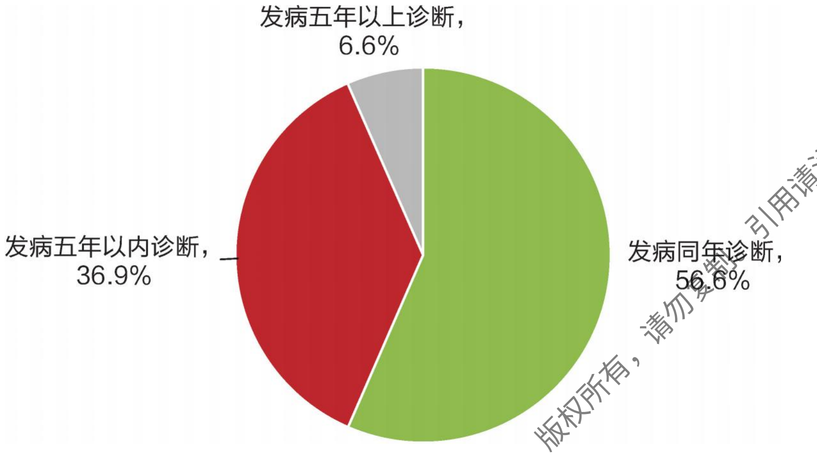
2）发病年龄与诊断年龄

受访者中 21-30 岁年龄段发病的人数最多，共 320 人，占比 31.4%；其次是 31-40 岁的年龄段，共有 202 名患者，占比 19.8%；70 岁以上年龄段发病的患者数量最少，共 3 人，占比 0.3%。 患者平均发病年龄为 29.4 岁，中位数发病年龄为 28 岁。

受访者中 21-30 岁年龄段被诊断为重症肌无力的人数最多，共 336 人，占比 32.9%；其次是 31-40 岁的年龄段，共有 216 名患者，占比 21.2%；70 岁以上年龄段被诊断的患者数量最少，共 4 人，占比 0.4%。 患者平均被诊断为重症肌无力时的年龄为 31.0 岁，中位数诊断年龄为 29 岁。

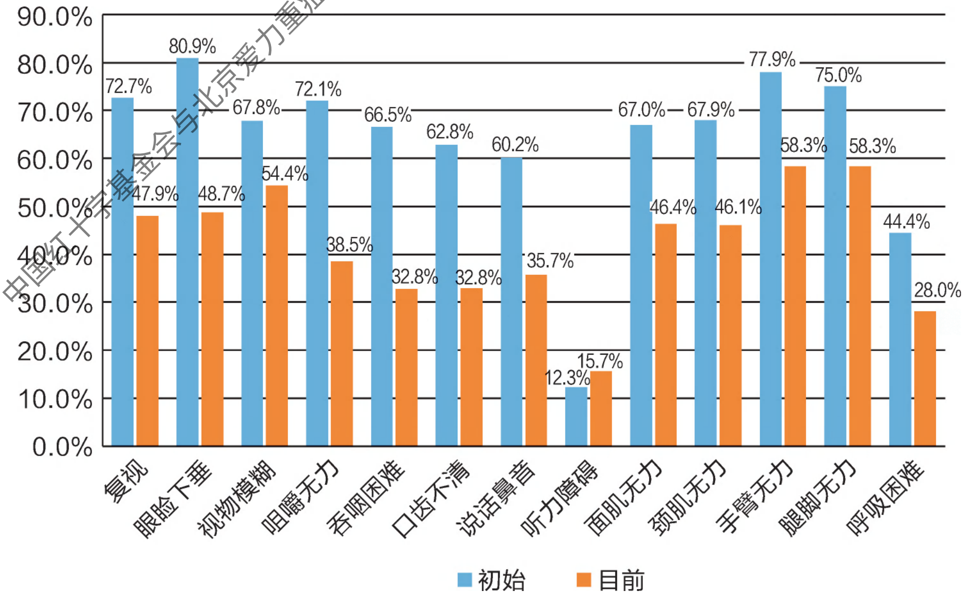


577 名患者在发病的同一年被诊断为重症肌无力，占比 56.6%；376 名患者在发病 1-5 年内确诊，占比 36.9%；67 名患者在发病 5 年以上才被确诊，占比 6.6%。



3）初始症状与目前症状

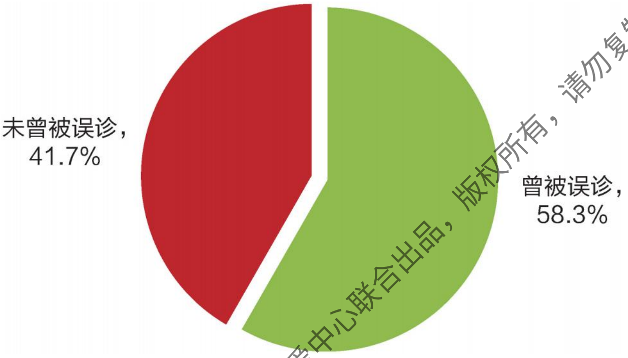
受访患者中最常见的初始症状为眼睑下垂，共 825 名患者，占比 80.9%；其次是手臂无力，共 795 名患者，占比 77.9%。 最不常见的初始症状为听力障碍，共 125 名患者，占比 12.3%；其次为呼吸困难，共 453 名患者，占比 44.4%。



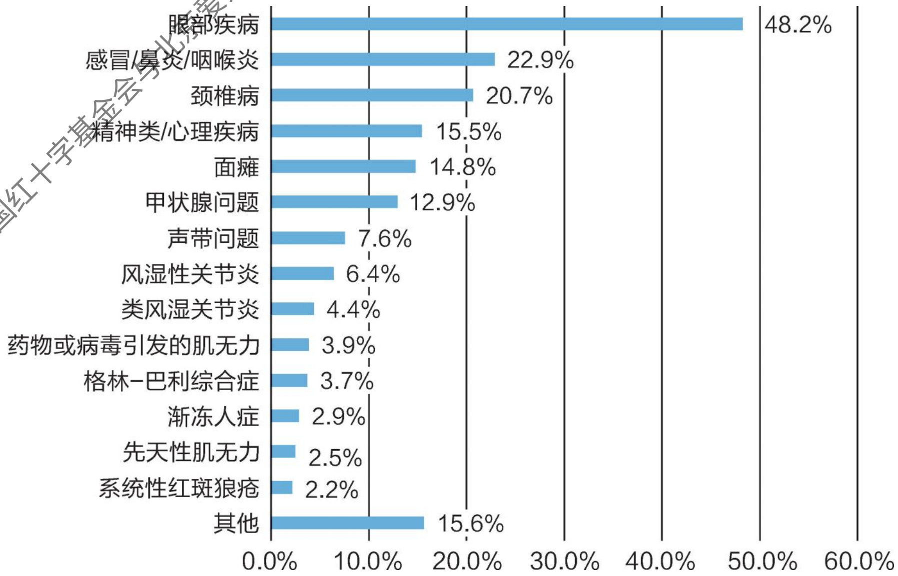
目前，受访患者中最常见的症状为手臂无力、腿脚无力，分别有 595 名患者，占比 58.3%；最不常见的目前症状为听力障碍，共 160 名患者，占比 15.7%；其次为呼吸困难，共 286 名患者，占比 28.0%。

4）误诊情况

受访患者中，共有 595 名患者曾经被误诊，占比 58.3%；425 名患者未曾被误诊，占比 41.7%。



在 595 名曾被误诊的患者中，被误诊为眼部疾病的患者最多，共 287 人，占比 48.2%；其次为感冒/鼻炎/咽喉炎，共 136 人，占比 22.9%；被误诊为系统性红斑狼疮的患者最少，共 13 人，占比 2.2%；其次为先天性肌无力，共 15 人，占比 2.5%。

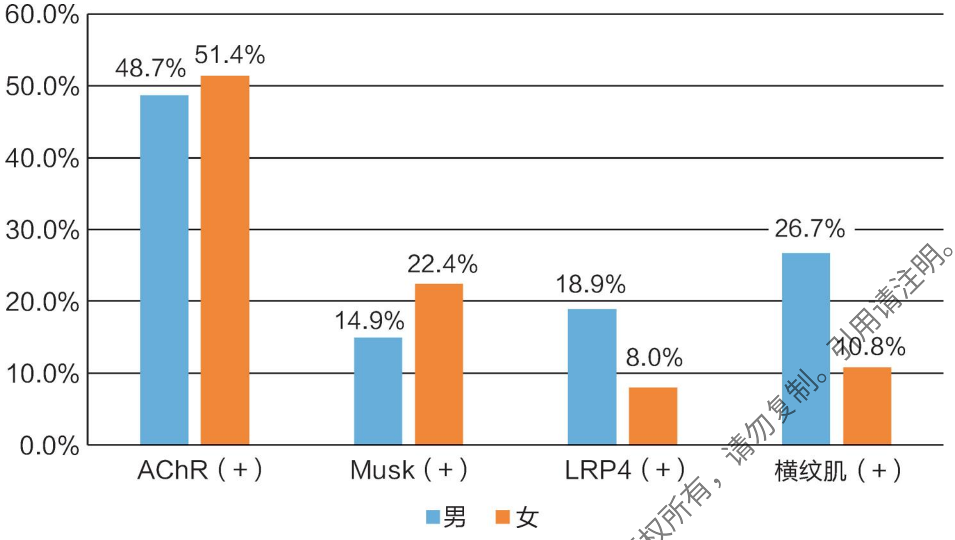


5）抗体检查

在受访患者中，586 人做过 AchR 抗体检查，占比 57.5%，其中 297 人为阳性，占比 50.7%；49 人为阴性，占比 8.4%。 279 人做过 Musk 抗体检查，其中 57 为阳性，占比 20.4%；86 人为阴性，占比 30.8%。 143 人做过 LRP4 抗体检查，其中 16 人为阳性，占比 11.2%；53 人为阴性，占比 37.1%。 138 人做过抗横纹肌抗体检查，其中 22 人为阳性，占比 15.9%；38 人为阴性，占比 27.5%。

类型	人数	%	结果					
			阳性	%	阴性	%	不清楚/不记得	%
AChR 抗体	586	57.5	297	50.7	49	8.4	240	41.0
Musk 抗体	279	27.4	57	20.4	86	30.8	136	48.8
LRP4 抗体	143	14.0	16	11.2	53	37.1	74	51.8
抗横纹肌抗体	138	13.5	22	15.9	38	27.5	78	56.5
都没做过	89	8.7	—					
不清楚	331	32.5	—					

做过 AChR 抗体测试患者中，男性阳性比例为 48.7%，低于女性阳性比例（51.4%）；Musk 抗体测试男性阳性比例为 14.9%，低于女性阳性比例（22.4%）；LRP4 抗体测试男性阳性比例为 18.9%，高于女性阳性比例（8.0%）；横纹肌抗体测试男性阳性比例为 26.7%，高于女性阳性比例（10.8%）。



6）诊断检测

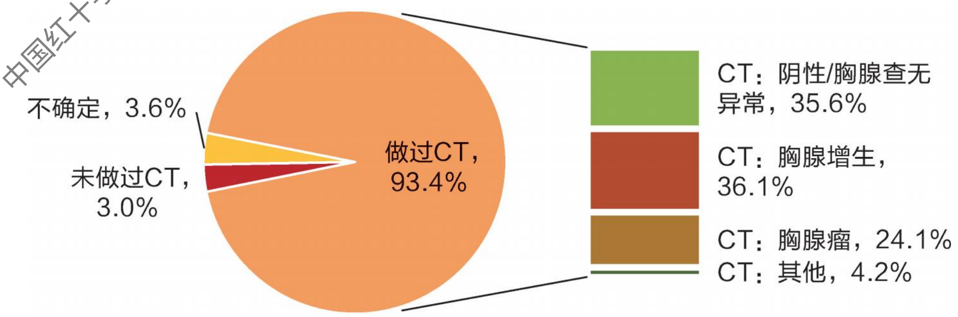
在受访患者中，839 人做过肌电图测试，占比 82.3%，其中 457 人为阳性，占比 54.5%；65 人为阴性，占比 7.7%。 754 人做过新斯的明试验，其中 524 为阳性，占比 69.5%；15 人为阴性，占比 2.0%。 323 人做过疲劳测试，其中 215 人为阳性，占比 66.6%；15 人为阴性，占比 4.6%。

类型	人数	%	结果					
			阳性	%	阴性	%	不清楚/不记得	%
肌电图	839	82.3	457	54.5	65	7.7	317	37.8
新斯的明试验	754	73.9	524	69.5	15	2.0	215	28.5
疲劳测试	323	31.7	215	66.6	15	4.6	93	28.8

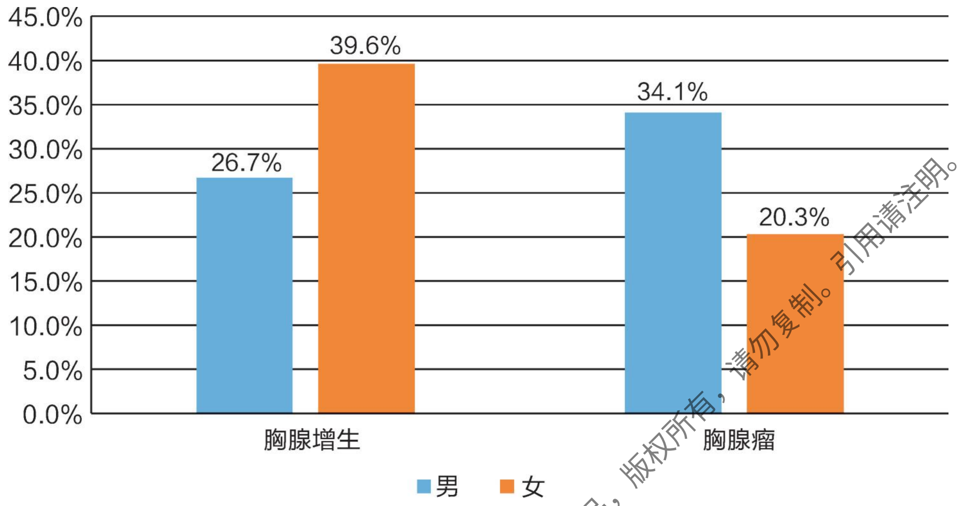
类型	人数	%	结果					
			阳性	%	阴性	%	不清楚/不记得	%
睡眠/休息测试	51	5.0	19	37.3	2	3.9	30	58.8
腾喜龙试验	26	2.6	18	69.2	1	3.8	7	26.9
冰敷测试	18	1.8	12	66.7	5	27.8	1	5.6
都没做过	14	1.4	——					
不清楚	59	5.8	——					

7）CT 检查

在受访患者中，952 人做过 CT 检查，占比 93.4%；31 人未做过 CT 检查，占比 3.0%。在做过 CT 检查的 952 名患者中，检查结果为胸腺增生的患者最多，有 344 人，占比 36.1%；其次为无异常的患者，有 339 人，占比 35.6%；229 名患者检查结果为胸腺瘤，占比 24.1%。



CT 结果为胸腺增生的男性患者比例为 26.7%，低于女性比例（39.6%）；结果为胸腺瘤的男性患者比例为 34.1%，高于女性比例（20.3%）。

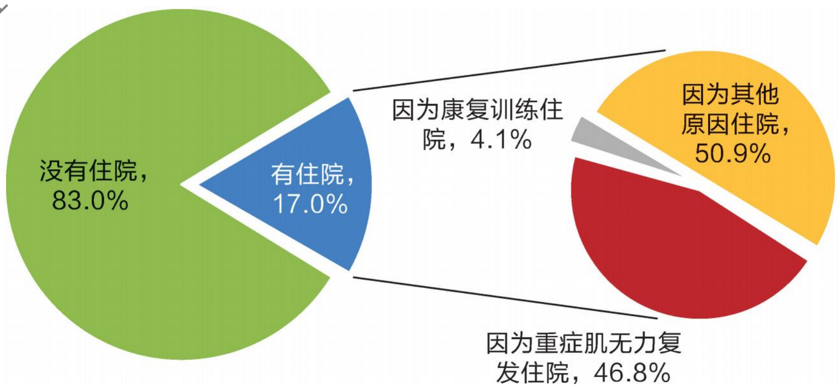


6. 住院与就诊经历

1）住院治疗

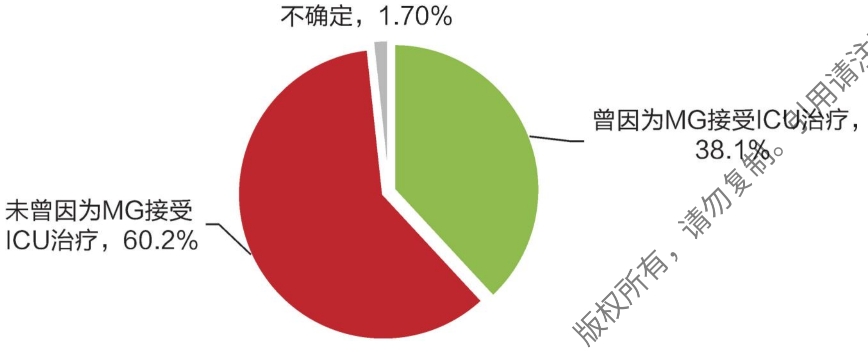
在过去六个月内，173 名患者曾经住院，占比 17.0%；847 名患者未曾住院，占比 83.0%。 在曾经住院的 173 名患者中，81 人曾因为重症肌无力复发而住院，占比 46.8%；7 人曾因为康复训练住院，占比 4.1%。

在曾因为其他原因住院的 88 名患者中，6 名患者因为复查住院（6.8%），6 名患者因为常规治疗住院（6.8%）。 4 名患者因为感冒住院（4.5%），3 名患者因为并发症住院（3.4%）。

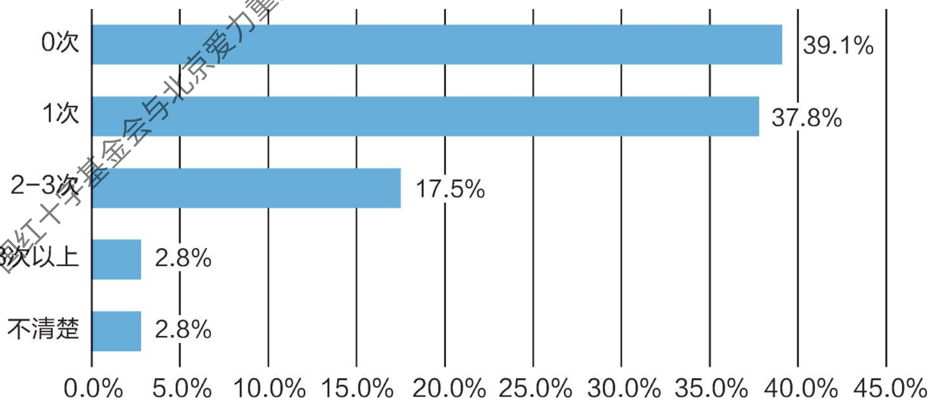


2）ICU 治疗与危象

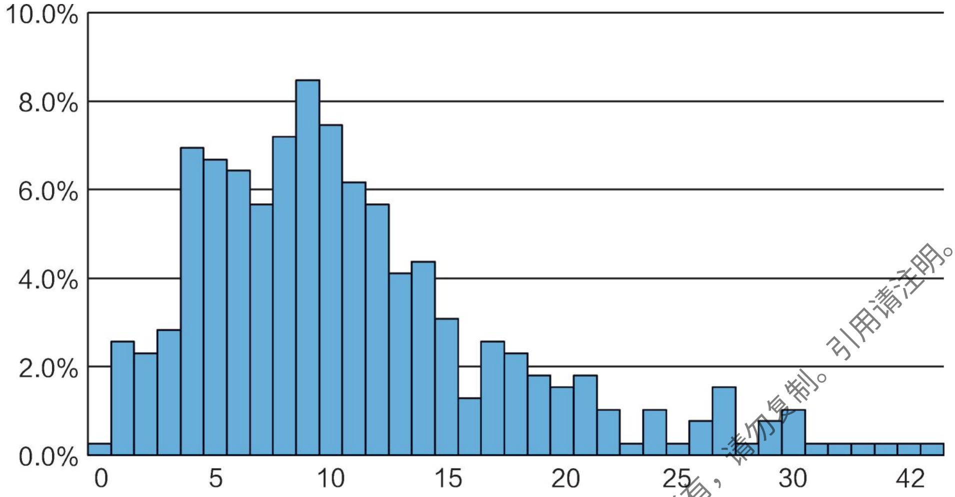
在受访患者中，389 人曾因为重症肌无力接受 ICU 治疗，占比 38.1%；614 人未曾因为重症肌无力接受 ICU 治疗，占比 60.2%。



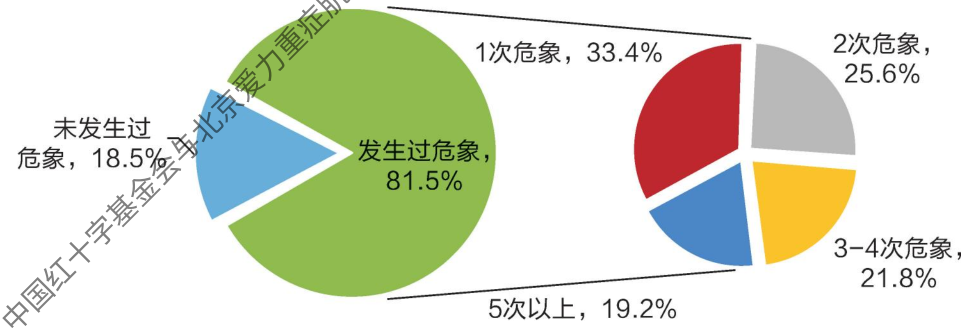
在 389 名曾因为重症肌无力接受 ICU 治疗的患者中，过去五年内，152 人未进行 ICU 治疗，占比 39.1%；147 人曾有一次 ICU 治疗，占比 37.8%；68 人曾有 2-3 次 ICU 治疗，占比 17.5%；11 人曾有 3 次以上 ICU 治疗，占比 2.8%。



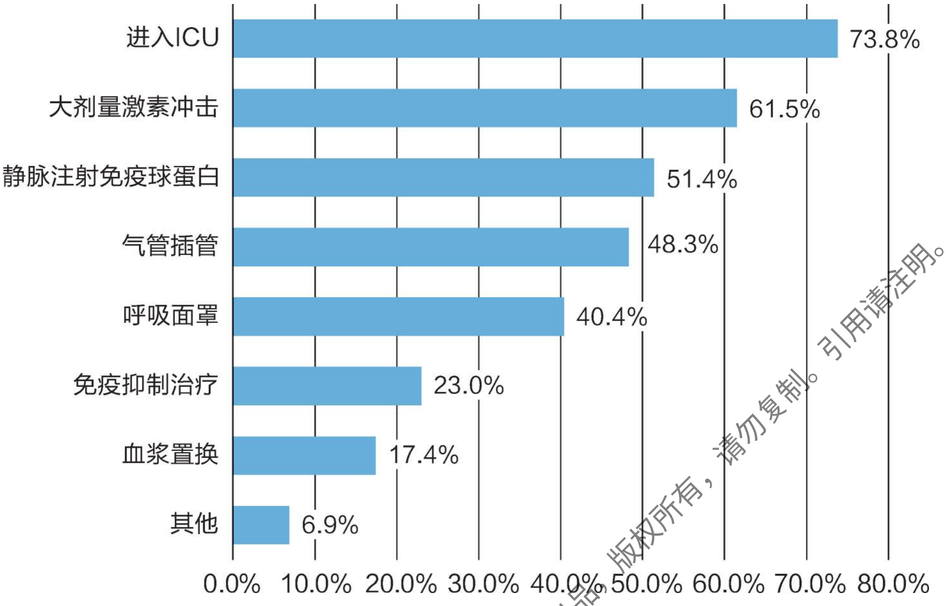
下图为五年内接受过 ICU 治疗的 389 名患者的病程时间分布。 病程在 4-10 年间的患者比例最高，占 48.8%。 病程在 15 年以上的患者比例较低。



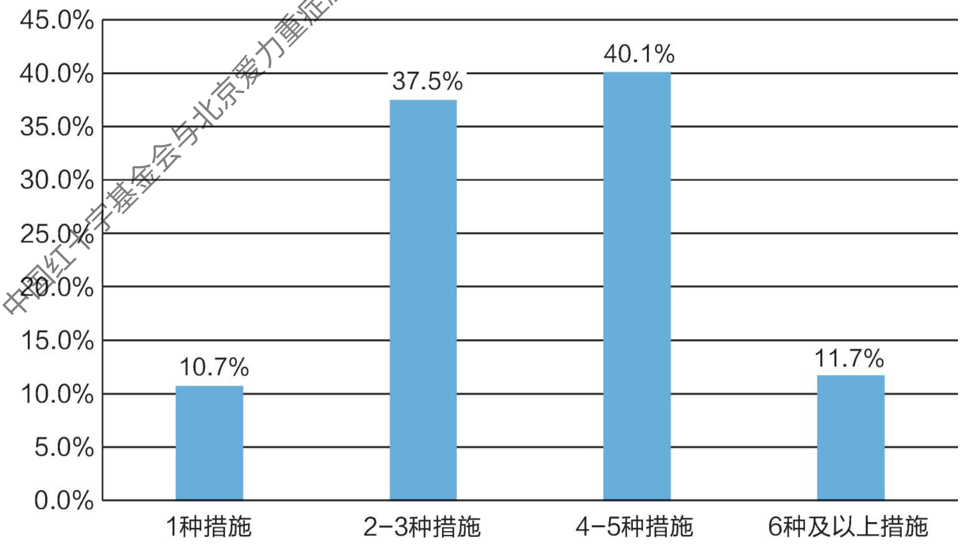
在曾在 ICU 接受治疗的 389 名受访患者中，317 人曾发生过危象，占比 81.5%；72 人未曾发生过危象，占比 18.5%，其中发生过 1 次危象的人最多，共 106 人，占比 33.4%，其次为 2 次危象，共 81 人，占比 25.6%，有 61 人发生五次及以上危象，共 61 人，占比 19.2%。



在 317 名曾发生危象的患者中，最常见的抢救措施为进入 ICU 治疗，共有 234 名患者，占比 73.8%；其次大剂量激素冲击，共有 153 名患者，占比 61.5%；最不常见的抢救措施为血浆置换，共有 55 名患者，占比 17.4%。

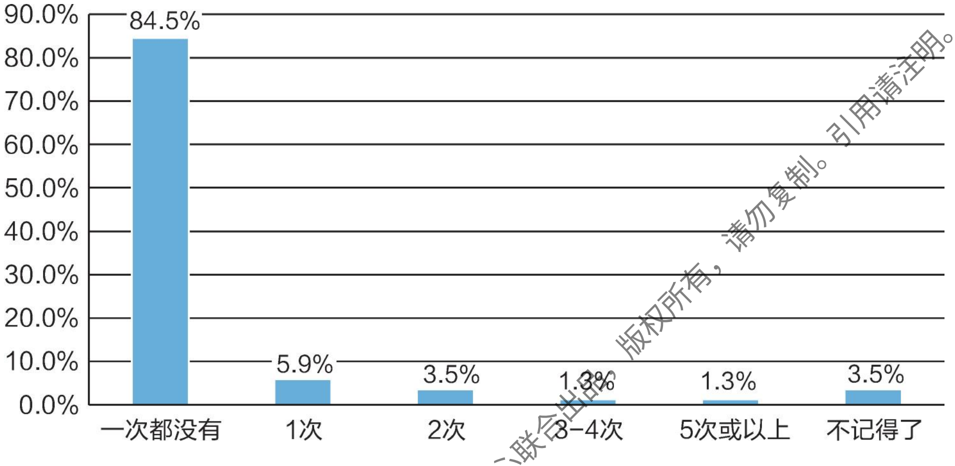


发生危象后，10.7%的患者接受了一种处理措施，37.5%的患者接受了2-3种处理措施，40.1%的患者接受了4-5种措施，11.7%的的患者接受了6种及以上措施。



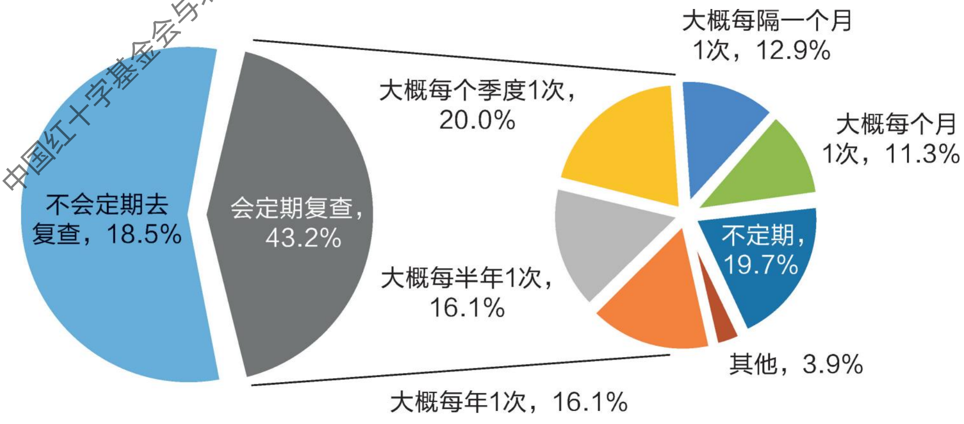
3）急诊治疗

在过去六个月内，862 名患者未曾看过急诊，占比 84.5%；60 名患者看过 1 次急诊，占比 5.9%；13 名患者看过至少 5 次急诊，占比 1.3%。



4）复查

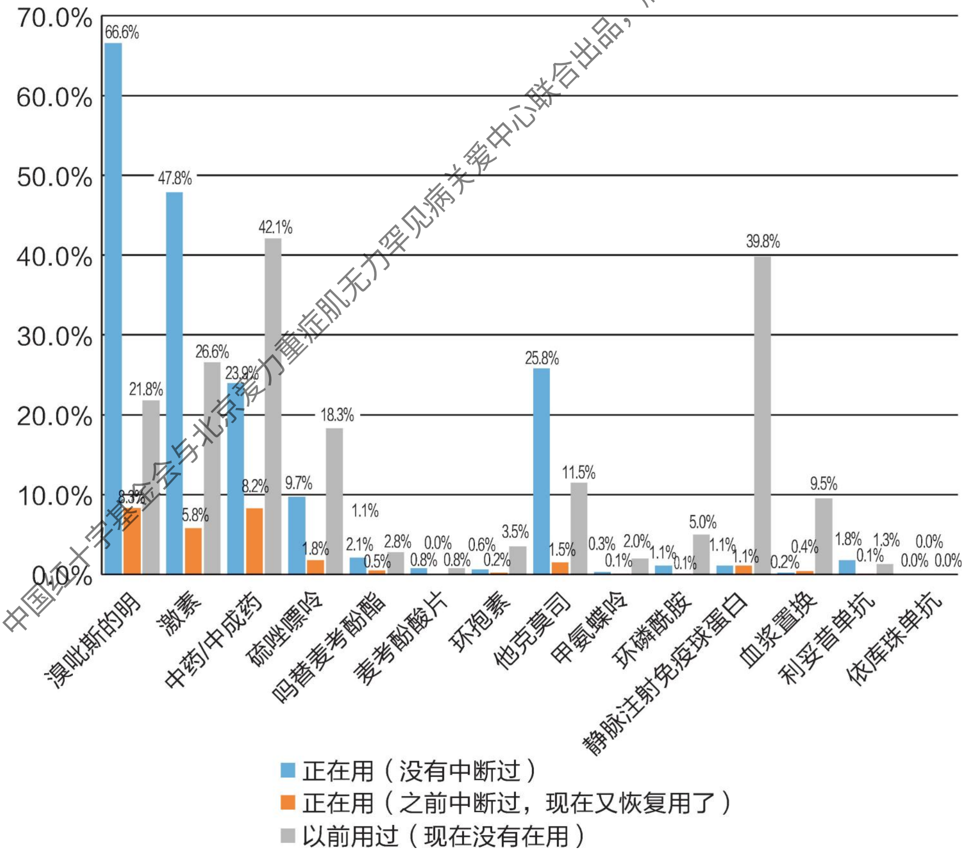
受访患者中，441 人在确诊后会定期到医院随诊或复查，占比 43.2%。其中，频率为每季度复查 1 次的患者最多，共 88 人，占 20.0%；其次为不定期复查的患者，共 87 人，占比 19.7%。



7. 药物治疗

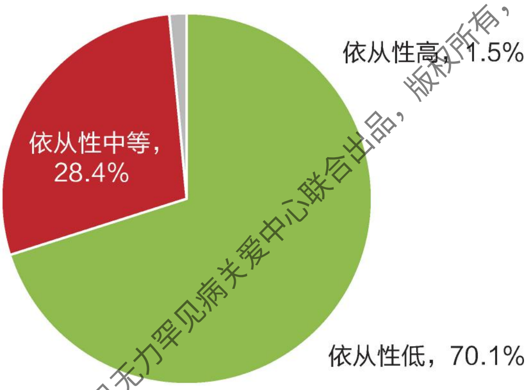
1）药物治疗与使用

在受访患者中，正在使用溴吡斯的明的患者最多，共 679 人，占比 66.6%；其次为激素类药物，共 488 人，占比 47.8%。 正在使用甲氨蝶呤的患者数最少，共 3 人，占比 0.3%。



2）服药依从性 - MMAS-8

本次调研运用了 MMAS-8 依从性测量量表评估了用药患者的服药依从性（量表参见附录），量表共 8 个条目，包括了用于测量患者有意不依从和无意不依从等方面的题目；量表总分为 0-8 分，分值越高，代表服药依从性越高。结果显示，在 946 名患者中，MMAS-8 分值范围为 0.25-8.00 分，平均值为 4.70 分，中位数为 4.75 分。仅有 1.5% 的患者服药依从性为高（8 分）；28.4% 的患者服药依从性中等（6-＜8 分）；70.1% 的患者服药依从性低（＜6 分）。



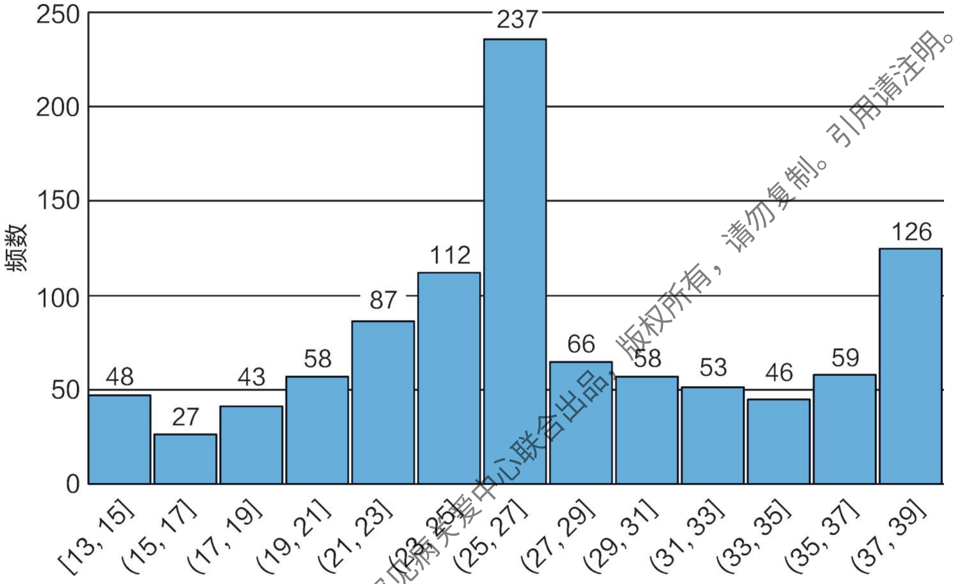
平均值	中位数	最小值	最大值
4.70	4.75	0.25	8.00

3）服药依从性 - SEAMS

本次调研运用了合理用药自我效能量表（SEAMS）评估了患者的服药依从性（量表参见附录），量表基于自我效能研制，具有良好的信效度且适合各种类型的慢性病患者。量表包含两个维度（不确定情况下和困难情况下的服药情况），共 13 个条目，总分范围为 13-39 分，分值越高，代表服药依从

性越高。

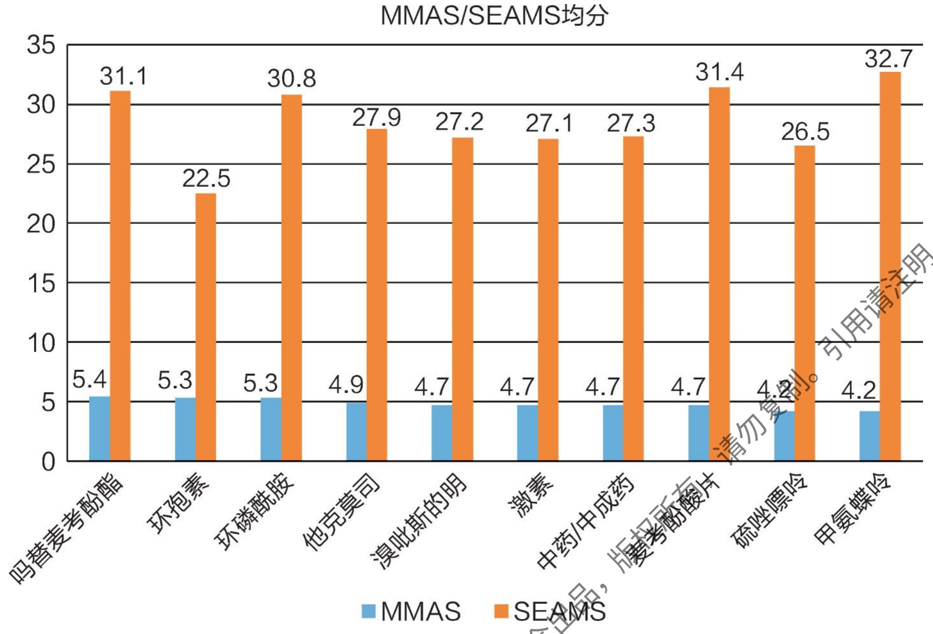
结果显示，在 1020 名患者中， SEAMS 分值范围为 13-39 分，平均值为 27.5 分，中位数为 26 分。 该分值越高，患者对于自我用药管理的信心越高。



平均值	中位数	最小值	最大值
27.5	26	13	39

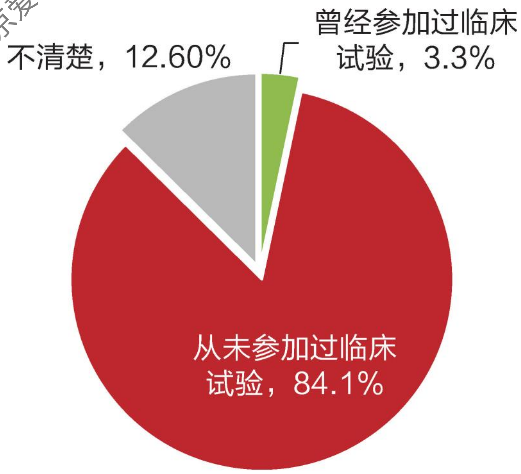
4）各类用药患者服药依从性

所有正在使用药物的患者中，正在使用吗替麦考酚酯的患者的 MMAS 均分最高，其次是使用环孢素和环磷酰胺的患者；硫唑嘌呤和甲氨蝶呤的患者 MMAS 均分最低。 从 SEAMS 均分来看，使用甲氨蝶呤和麦考酚酸片的患者平均分最高，使用环孢素和硫唑嘌呤的患者平均分最低。

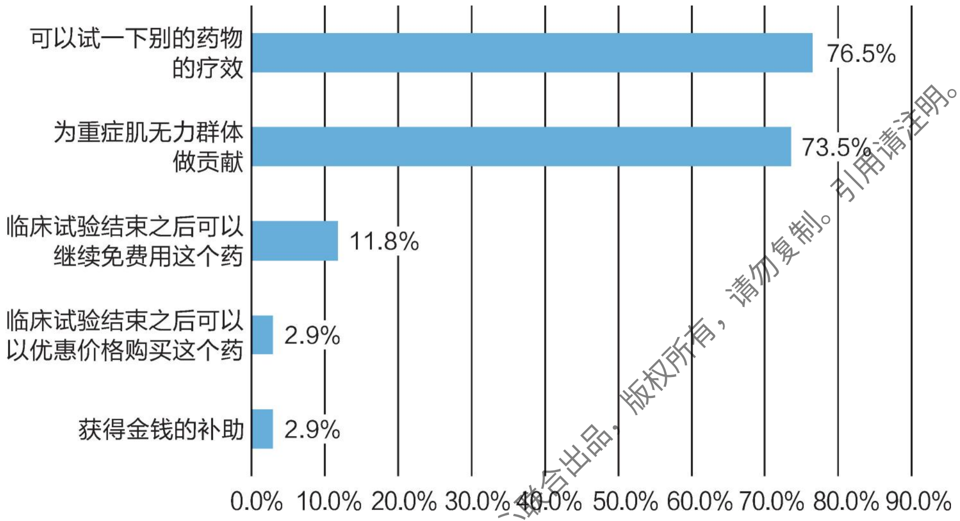


5）参与药物临床试验情况

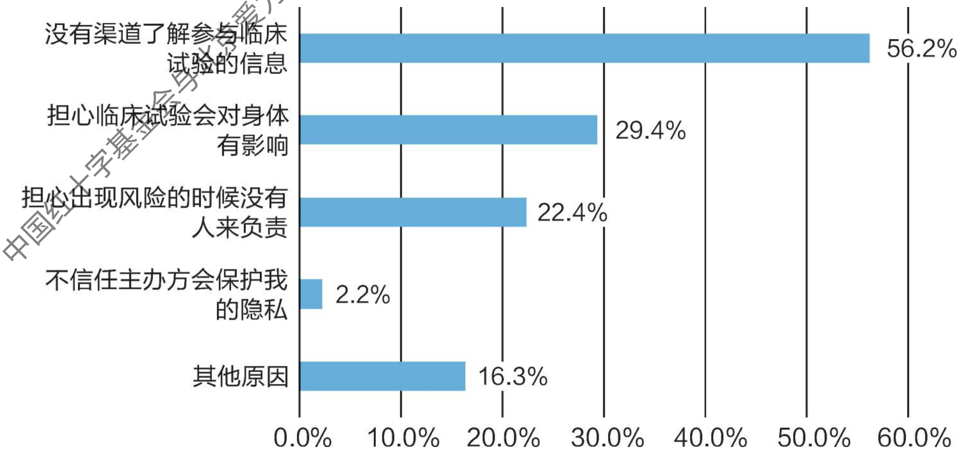
在受访患者中，34 人曾经参与过重症肌无力药物的临床试验，占比 3.3%；858 人从未参与过重症肌无力药物的临床试验，占比 84.1%。



在曾经参加过药物临床试验的34患者中，76.5%的患者认为参与临床试验的好处是试验药物疗效；73.5%的患者认为参与临床试验的好处是为重症肌无力群体做贡献。



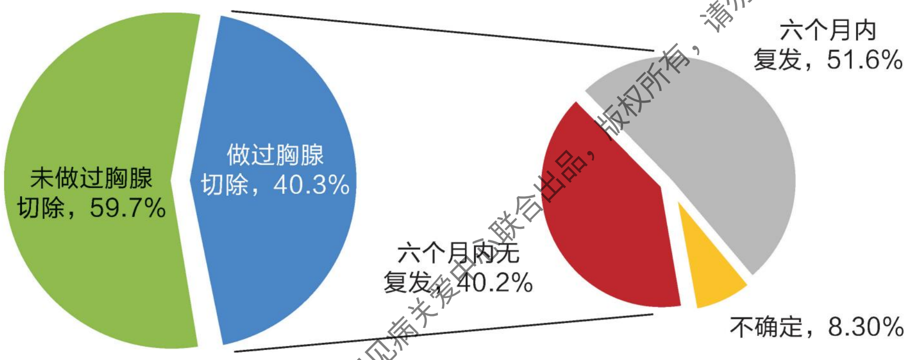
在从未参加过药物临床试验的858患者中，最常见的未曾参与的原因是缺乏了解临床试验的渠道，共482名患者，占比56.2%；其次原因为担心临床试验对身体的影响，共252人，占比29.4%。



8. 手术及其他治疗

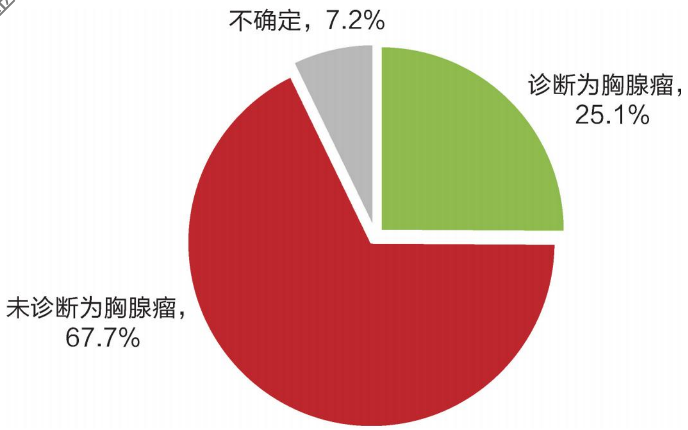
1）胸腺增生切除术

在受访患者中，411 人做过胸腺切除，占比 40.3%；609 人未做过胸腺切除，占比 59.7%。 在做过胸腺切除的 411 名患者中，212 人在术后六个月内经历病情复发或加重，占比 51.6%；165 人在六个月内未经历病情复发或加重，占比 40.2%。

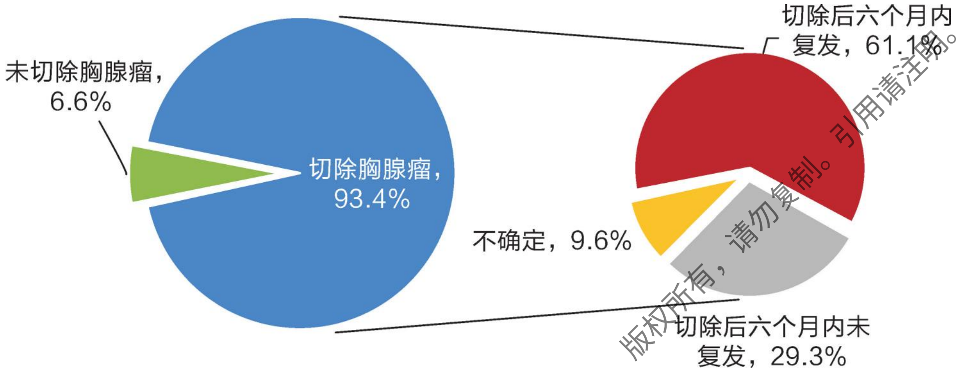


2）胸腺瘤切除术

在受访患者中，256 人曾被诊断为胸腺瘤，占比 25.1%；691 人未曾被诊断为胸腺瘤，占比 67.7%。

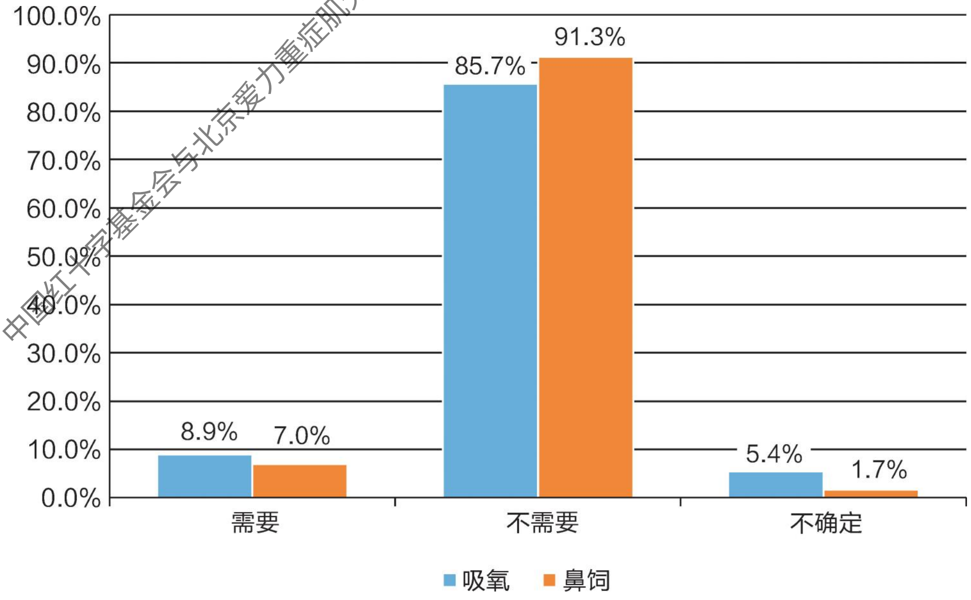


在诊断为胸腺瘤的256名受访患者中，239人做过胸腺瘤切除，占比93.4%；17人未做过胸腺切除，占比6.6%。在做过胸腺切除的239名患者中，146人在术后六个月内经历病情复发或加重，占比61.1%；70人在六个月内未经历病情复发或加重，占比29.3%。



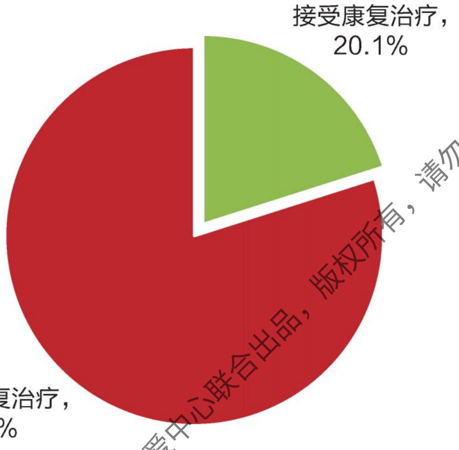
3）吸氧/胃肠鼻饲

在受访患者中，91人目前需要接受吸氧治疗，占比8.9%；71人目前需要使用胃肠管进食，占比7.0%。

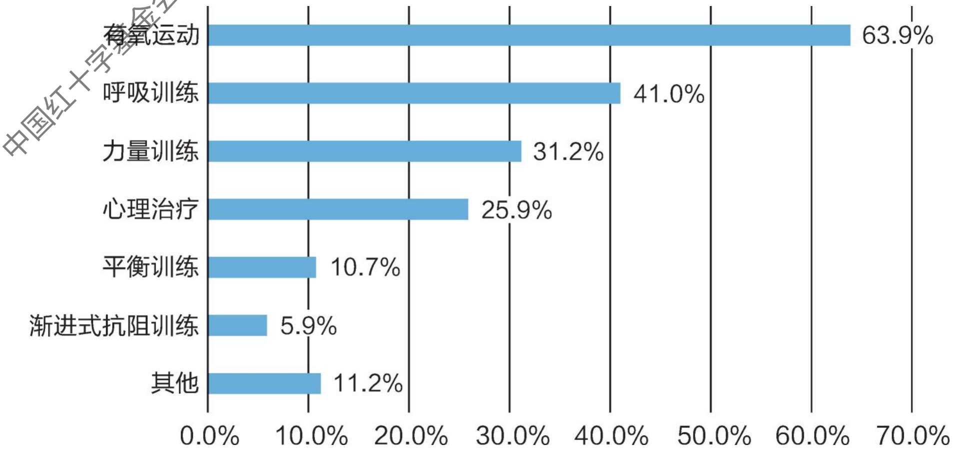


4）康复治疗

在受访患者中，205 人接受过康复治疗，占比 20.1%；815 人未接受康复治疗，占比 79.9%。

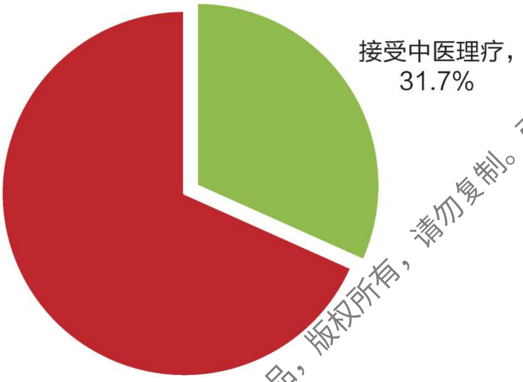


在接受康复治疗的 205 人中，最常见的康复治疗是有氧运动，共有 131 名患者，占比 63.9%；其次为呼吸训练，共有 84 名患者，占比 41.0%；最不常见的康复治疗是渐进式抗阻训练，共有 12 名患者，占比 5.9%。

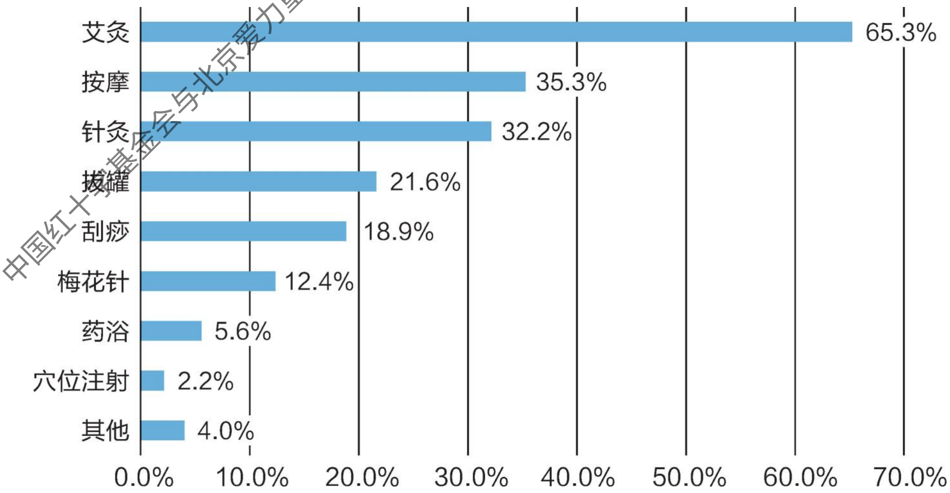


5）中医理疗

在受访患者中，323 人接受过中医理疗，占比 31.7%；697 人未接受中医理疗，占比 68.3%。

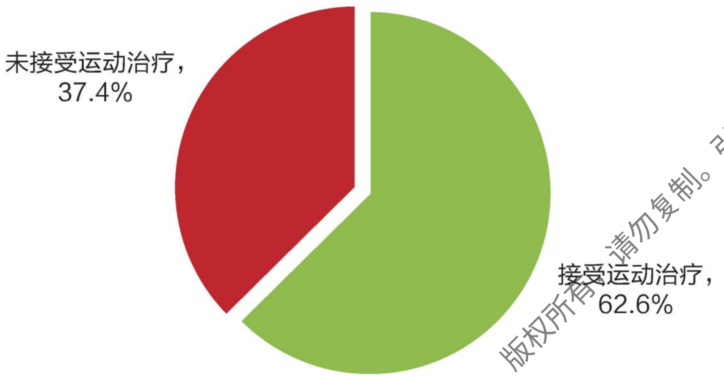


在 323 名接受中医理疗患者中，最常见的中医理疗是艾灸，共有 211 名患者，占比 65.3%；其次为按摩，共有 114 名患者，占比 35.3%；最不常见的中医理疗是穴位注射，共有 7 名患者，占比 2.2%。

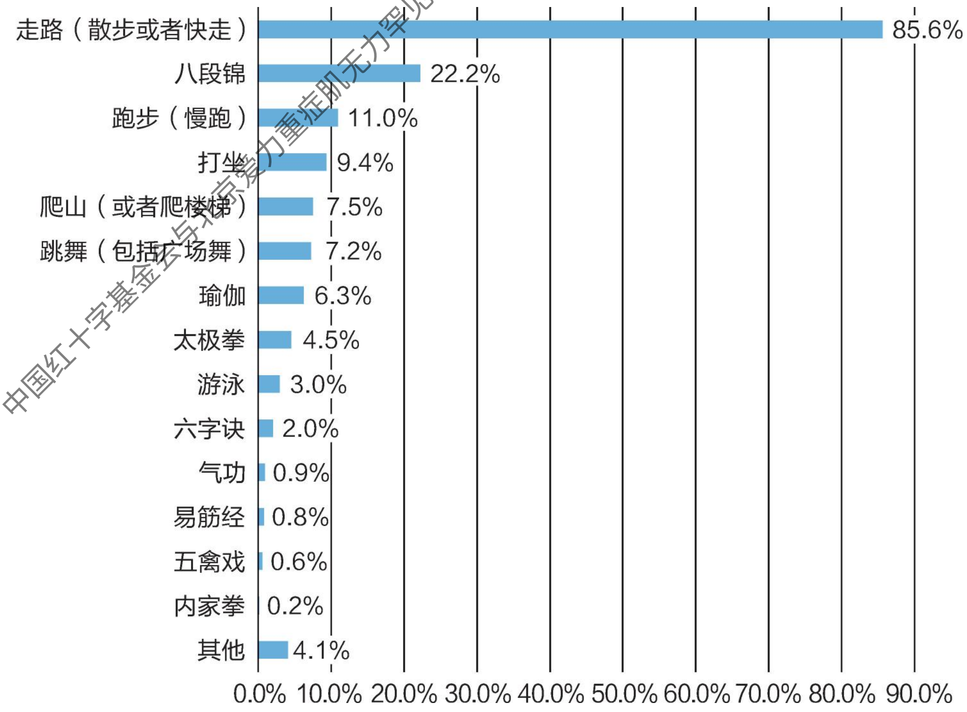


6）运动治疗

在受访患者中，639 人接受过运动治疗，占比 62.6%；381 人未接受运动治疗，占比 37.4%。



在 639 名接受运动治疗的患者中，最常见的运动治疗是走路，共有 547 名患者，占比 85.6%；其次为八段锦，共有 142 名患者，占比 22.2%；最不常见的运动治疗是内家拳，共有 1 名患者，占比 0.2%。

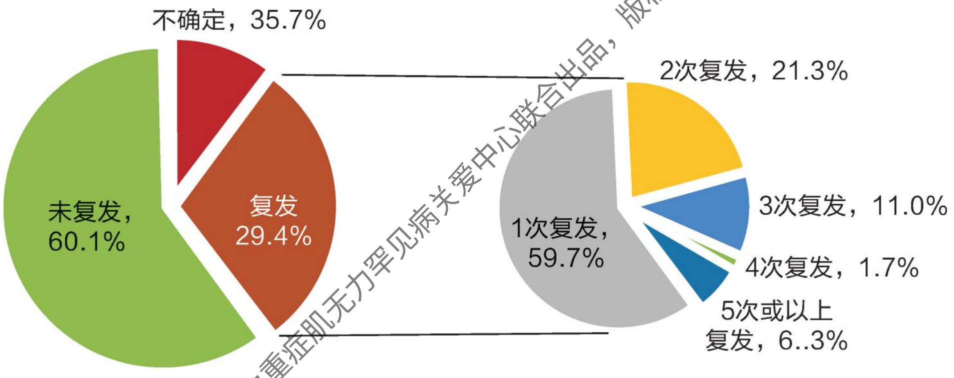


9. 复发与病情变化

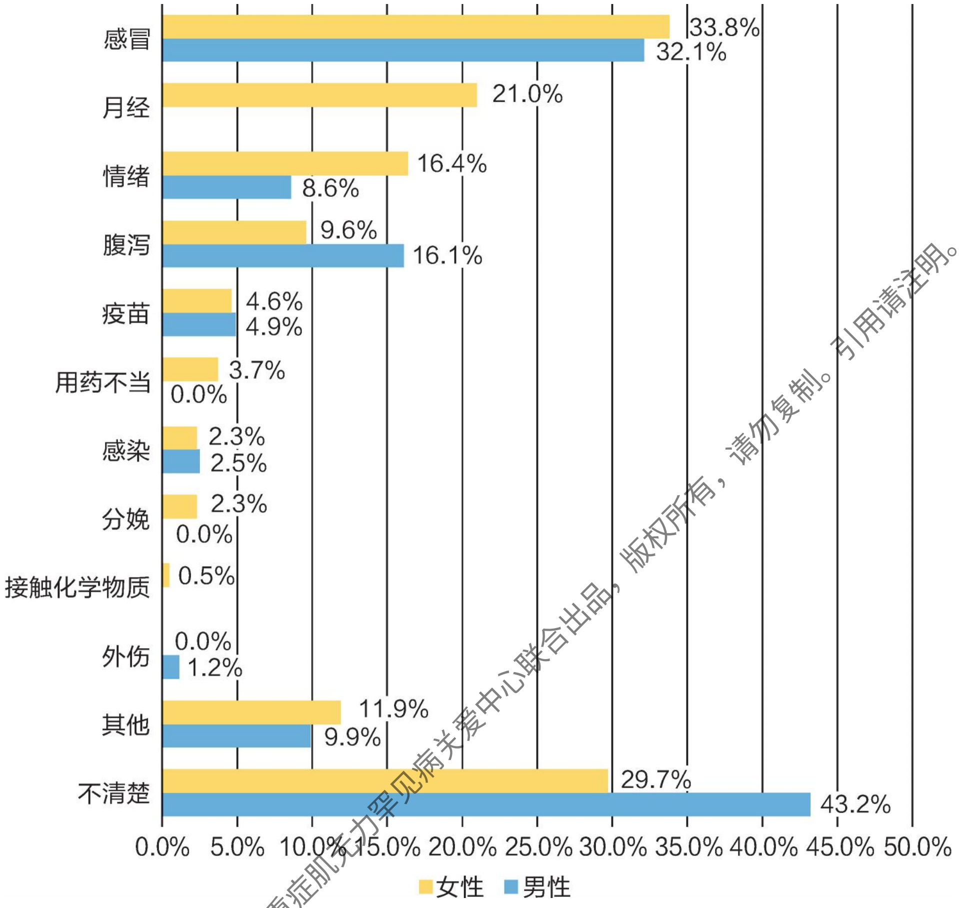
1）复发情况与复发原因

在过去六个月内，300 名患者病情复发，占比 29.4%；613 名患者病情无复发，占比 60.1%。

在 300 名过去六个月内有病情复发的患者中，1 次复发的患者最多，共 179 人，占比 59.7%；4 次复发的患者最少，共 5 人，占比 1.7%。

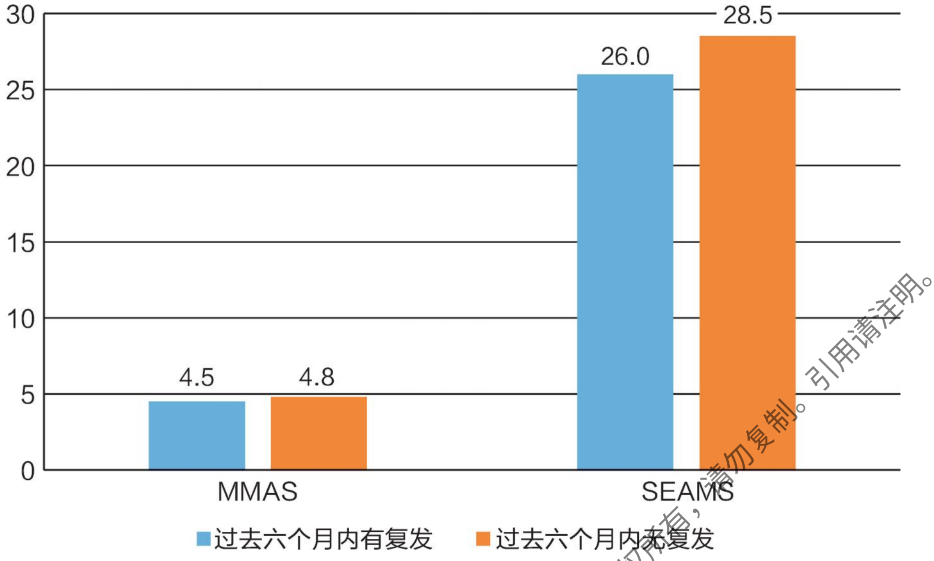


在 300 名过去六个月内有病情复发的患者中，女性患者为 219 人，男性患者为 81 人。 女性患者中，最常见的复发原因为感冒，共 74 人，占比 33.8%；其次为月经，共 46 人，占比 21.0%；最不常见的原因为接触化学物质，共 1 人，占比 0.5%；由外伤引起复发的女性患者为 0 人。 男性患者中，最常见的复发原因为感冒，共 26 人，占比 32.1%；其次为腹泻，共 13 人，占比 16.1%；最不常见的原因为接触化学物质，占比 0%。因为情绪原因引起病情复发的女性患者占 16.4%；男性患者中比例较低，占 8.6%。



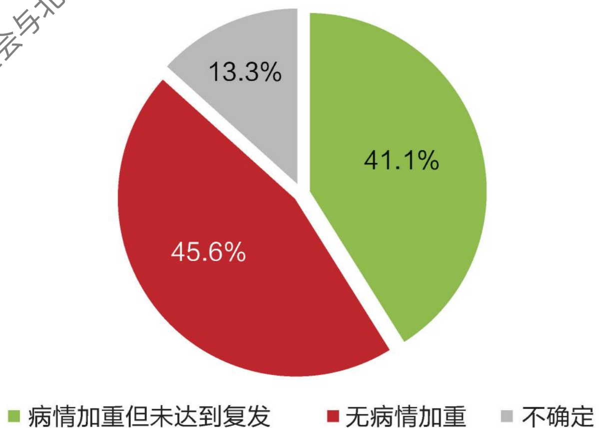
2）复发与用药依从性

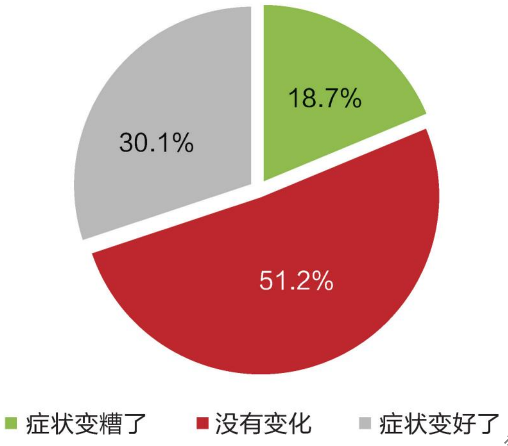
当比较复发患者与无复发患者的用药依从性时，结果显示，过去六个月内有复发的患者 MMAS 均分为 4.5 分，SEAMS 均分为 26.0 分；六个月内无复发的患者 MMAS 均分为 4.8 分，SEAMS 均分为 28.5 分。有复发患者的两类得均分较无复发患者低，且两者差值具有统计学意义。此结果提示用药依从性与病情复发存在初步相关性，但其实际关联需在进一步分析中验证。



3）病情变化

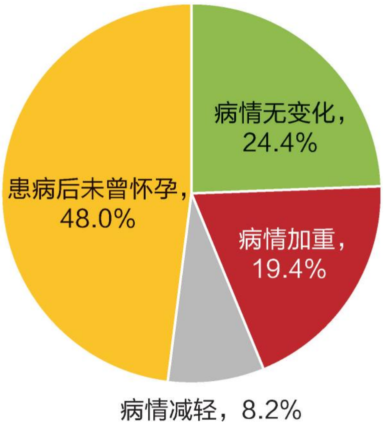
在过去六个月内，419 名患者病情发生加重，但未达到复发程度，占比 41.1%；465 名患者病情未发生加重，占比 45.6%。 191 名患者认为，和六个月之前相比，目前的症状变糟了，占比 18.7%；522 名患者认为症状无变化，占比 51.2%；307 名患者认为症状变好了，占比 30.1%。





4）女性孕产与病情

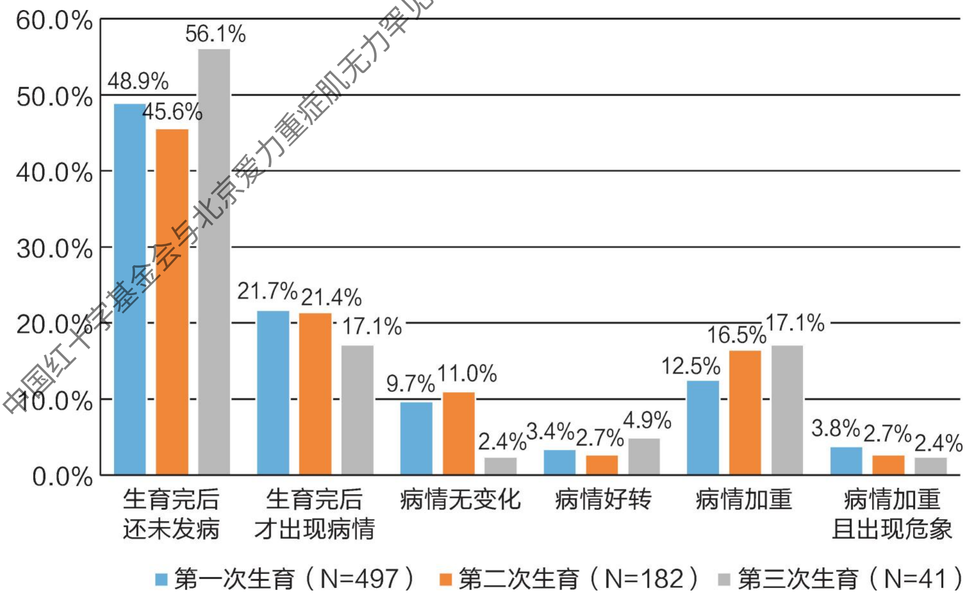
受访患者中，有537名女性患者曾经怀孕，其中，131名患者怀孕期间重症肌无力病情未发生变化，占比24.4%；104名患者在怀孕期间病情加重，占比19.4%；44名患者在怀孕期间病情减轻，占比8.2%。



受访患者中，共有 497 名经历女性患者经历过生育。 其中，62 名患者在第一次生育后病情加重但未发生危象，占比 12.5%；19 名患者病情加重且发生危象，占比 3.8%；48 名患者在病情无变化，占比 9.7%；17 名患者病情减轻，占比 3.4%。

在 182 名经历第二次生育的女性患者中，30 名患者在 第二次生育后病情加重但未发生危象，占比 16.5%；5 名患者病情加重且发生危象，占比 2.7%；20 名患者在病情无变化，占比 11.0%；5 名患者病情减轻，占比 2.7%。

在 41 名经历第三次生育的女性患者中，7 名患者在第三次生育后病情加重但未发生危象，占比 17.1%；1 名患者病情加重且发生危象，占比 2.4%；1 名患者在病情无变化，占比 2.4%；2 名患者病情减轻，占比 4.9%。

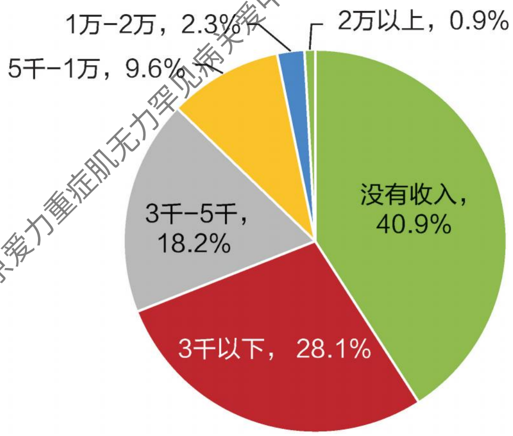


10. 患者经济状况

1）患者个人月收入

所有受访患者中，417 人目前没有收入，占比 40.9%；287 人月收入在 3000 元以下，占比 28.1%；186 人月收入为 3000-5000 元，占比 18.2%；98 人月收入为 5000-1 万元，占比 9.6%；23 人月收入为 1 万-2 万，占比 2.3%；9 人月收入在 2 万以上，占比 0.9%。

在 603 名有收入的患者中，个人月收入范围在 119 元-10 万元之间，平均月收入为 4426 元，中位数为 3000 元。

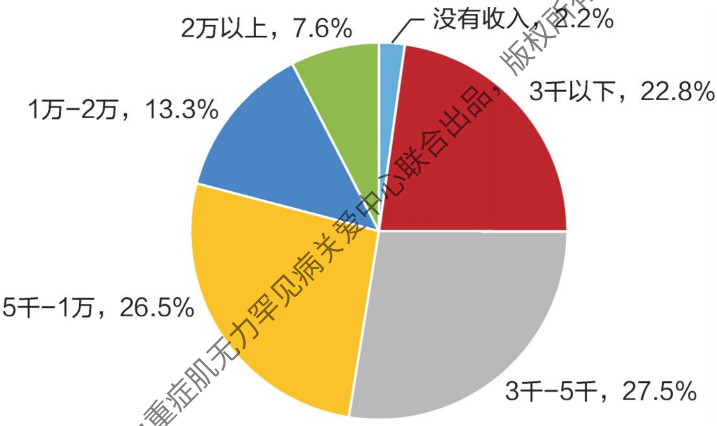


平均值	中位数	最小值	最大值
4,426	3,000	119	100,000

2）患者家庭总月收入

所有受访患者中，22 人目前没有家庭总月收入，占比 2.2%；233 人家庭月收入在 3000 元以下，占比 22.8%；281 人月收入为 3000-5000 元，占比 27.5%；270 人月收入为 5000-1 万元，占比 26.5%；136 人月收入为 1 万-2 万，占比 13.3%；78 人月收入在 2 万以上，占比 7.6%。

在 998 名有家庭月收入的患者中，家庭总月收入范围在 110 元-30 万元之间，平均家庭月收入为 10646 元，中位数为 5000 元。①



平均值	中位数	最小值	最大值
10,646	5,000	110	300,000

在扣除家庭日常生活月支出后，患者家庭可支配月收入范围在 0 元-29 万元之间，平均家庭月收入为 6503.8 元，中位数为 2000 元。

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① 注：如去除处于家庭总月收入最低与最高的 5%的患者，其余 682 名患者的家庭总月收入范围在 3008 元-2.7 万元之间，平均家庭月收入为 8532.9 元，中位数为 7000 元。

平均值	中位数	最小值	最大值
6503.8	2,000	0	290,000

11. 医疗支出

1）口服药物经济负担- 月支出

- 在使用溴吡斯的明的 764 名患者中，该药物的月支出范围在 0 元-5200 元之间，平均月支出为 207.4 元，中位数为 150 元。
- 在使用激素的 547 名患者中，该药物的月支出范围在 0 元-600 元之间，平均月支出为 51.6 元，中位数为 15 元。
- 在使用中药/中成药的 328 名患者中，该药物的月支出范围在 50 元-3 万元之间，平均月支出为 1911 元，中位数为 1500 元。
- 在使用他克莫司的 278 名患者中，该药物的月支出范围在 200 元-6000 元之间，平均月支出为 1366.3 元，中位数为 1200 元。
- 在使用硫唑嘌呤的 117 名患者中，该药物的月支出范围在 0 元-1 万元之间，平均月支出为 259.3 元，中位数为 108 元。
- 在使用吗替麦考酚酯的 26 名患者中，该药物的月支出范围在 350 元-2500 元之间，平均月支出为 1024.9 元，中位数为 1000 元。
- 在使用环磷酰胺的 11 名患者中，该药物的月支出范围在 0 元-3000 元之间，平均月支出为 512.3 元，中位数为 247.5 元。
- 在使用环孢素的 8 名患者中，该药物的月支出范围在 100 元-1600 元之间，平均月支出为 873.1 元，中位数为 750 元。
- 在使用麦考酚酸片的 8 名患者中，该药物的月支出范围在 330 元-

1600 元之间，平均月支出为 948 元，中位数为 890 元。

- 在使用甲安蝶呤的 4 名患者中，该药物的月支出范围在 30 元-90 元之间，平均月支出为 52.5 元，中位数为 45 元。

药物名称	使用人数	支出/月			
		平均值	中位数	最小值	最大值
溴吡斯的明	763	207.4	150	0	5,200
激素	547	51.6	15	0	600
中药/中成药	328	1911.0	1,500	50	30,000
他克莫司	278	1366.3	1,200	200	6,000
硫唑嘌呤	117	259.3	108	0	10,000
吗替麦考酚酯	26	1024.9	1,000	350	2,500
环磷酰胺	11	512.3	247.5	0	3,000
环孢素	8	873.1	750	100	1,600
麦考酚酸片	8	948.0	890	330	1,600
甲氨蝶呤	4	52.5	45	30	90

2）用药药物经济负担- 单次使用支出

在使用过静脉注射免疫球蛋白的 22 名患者中，该药物单次使用的支出范围在 8 元-5.3 万元之间，平均月支出为 25609.1 元，中位数为 3 万元。

在使用过血浆置换的 6 名患者中，该药物单次使用的支出范围在 8000 元-5 万元之间，平均月支出为 26817.7 元，中位数为 2.2 万元。

在使用过利妥昔单抗的 19 名患者中，该药物单次使用的支出范围在 3000 元-4 万元之间，平均月支出为 12497.9 元，中位数为 1.2 万元。

药物名称	使用人数	支出/次			
		平均值	中位数	最小值	最大值
免疫球蛋白	22	25609.1	30,000	8	53,000
血浆置换	6	26817.7	22,000	8,000	50,000
利妥昔单抗	19	12497.9	12,000	3,000	40,000
依库珠单抗	0	—	—	—	—

3）药物费用占家庭月收入的比例

口服用药支出占患者个人或家庭月收入比例最高的药物为中药/中成药，月支出占患者个人月收入的中位数比例为 43.9%，占家庭月收入的比例中位数为 26.7%，占家庭月可支配收入的比例中位数为 50.0%；其次为他克莫司，月支出占患者个人月收入的中位数比例为 42.9%，占家庭月收入的比例中位数为 21.7%，占家庭月可支配收入的比例中位数为 50.0%。

用药支出占患者个人或家庭月收入比例最低的药物是激素，该药物的月支出占患者个人月收入的中位数比例为0.5%，占家庭月收入的比例中位数为0.3%，占家庭月可支配收入的比例中位数为0.8%；其次为在使用甲氨蝶呤患者中，该药物的月支出占患者个人月收入的中位数比例为2.3%，占家庭月收入的比例中位数为0.8%，占家庭月可支配收入的比例中位数为1.7%。

药物名称	月支出（中位数）	占个人月收入的比例（中位数）	占家庭月收入的比例（中位数）	占家庭月可支配收入的比例（中位数）
溴吡斯的明	150	4.0%	2.7%	6.0%
激素	15	0.5%	0.3%	0.8%
中药/中成药	1500	43.9%	26.7%	50.0%
他克莫司	1200	42.9%	21.7%	50.0%
硫唑嘌呤	108	3.4%	2.4%	5.2%
吗替麦考酚酯	1000	26.7%	21.8%	50.0%
环磷酰胺	247.5	6.7%	3.3%	7.8%
环孢素	750	19.9%	10.8%	25.8%
麦考酚酸片	890	34.3%	23.0%	66.5%
甲氨蝶呤	45	2.3%	0.8%	1.7%

4）过去一年总体医疗支出情况

在所有患者中，过去 12 个月的直接医疗费用中位数为 12742.5 元，间接医疗费用中位数为 1700 元，可报销费用中位数为 0 元，个人自付金额中位数为 14400 元。

医疗支出	中位数（元）	下四分位数（元）	上四分位数（元）
支出费用			
直接医疗费用	12742.5	3000	30000
间接医疗费用	1700	0	5000
误工费	0	0	1000
总计支出	16000	4950	39200
可报销的费用			
医保报销	0	0	3000
商业保险/众筹或者慈善捐赠	0	0	0
总计报销	0	0	3000
自付费用			
个人自付金额	14400	3650	32615

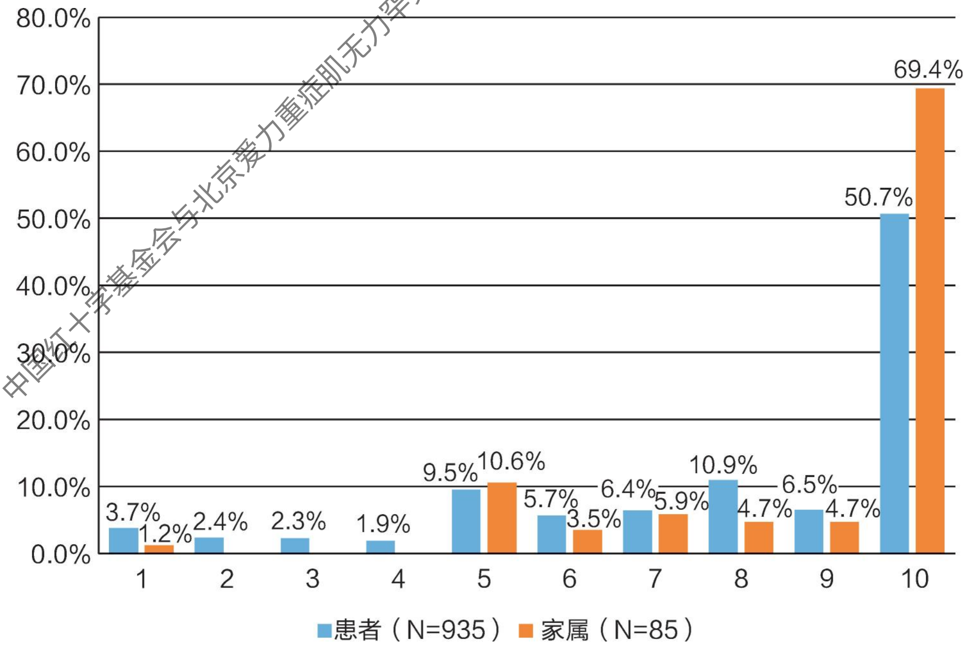
12. 新药支付意愿- 患者 vs 照顾者

本调查对自填问卷的 935 名的患者与代填问卷的 85 名患者照顾者对重症与无力新药的支付意愿进行调查。

1）对新药的迫切程度

本调查采取 1-10 分的量表测量患者和照顾者对重症肌无力新药的迫切程度，1 分代表迫切程度最低，10 分代表迫切程度最高。

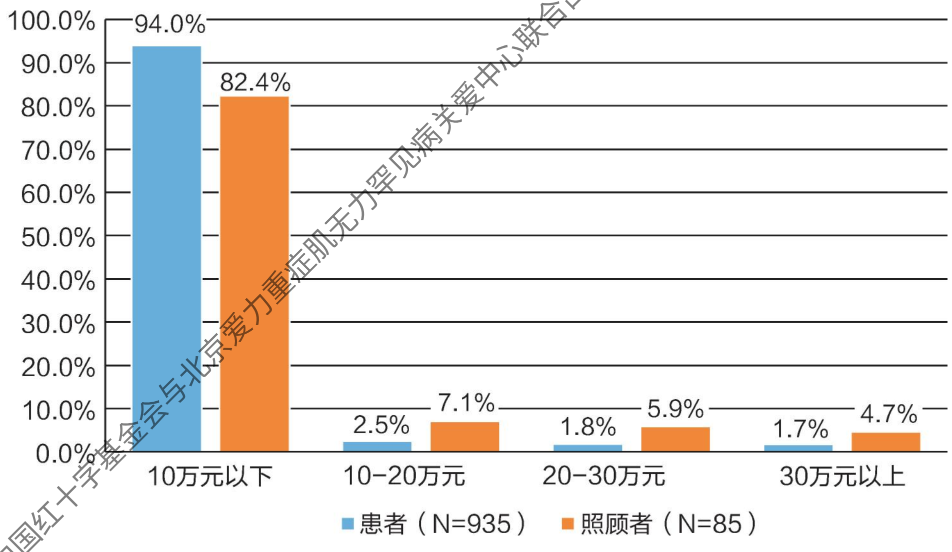
在 935 名患者中，50.7% 的患者目前对新药的迫切程度为非常高（10 分），3.7% 的患者目前对新药的迫切程度非常低（1 分）。 在 85 名患者照顾者中，69.4% 的照顾者目前对新药的迫切程度为非常高（10 分），1.2% 的照顾者目前对新药的迫切程度非常低（1 分）。



2）新药支付意愿

对于重症肌无力新药，879 名患者表示能接受的每年最高支付费用在 10 万元以下，占比 94.0%；23 名患者最高愿意支付在 10-20 万元，占比 2.5%；17 名患者最高愿意支付 20-30 万元，占比 1.8%；16 名患者最高愿意支付 30 万元及以上，占比 1.7%。

在患者照顾者中，70 名照顾者表示能接受的每年最高支付费用在 10 万元以下，占比 82.4%；6 名照顾者最高愿意支付在 10-20 万元，占比 7.1%；5 名照顾者最高愿意支付 20-30 万元，占比 5.9%；4 名照顾者最高愿意支付 30 万元及以上，占比 4.7%。



每年新药最高支付意愿在 10 万元以下的患者中，具体支付意愿的范围在 0-8 万元之间，平均值为 12204.5，中位数为 1 万元。

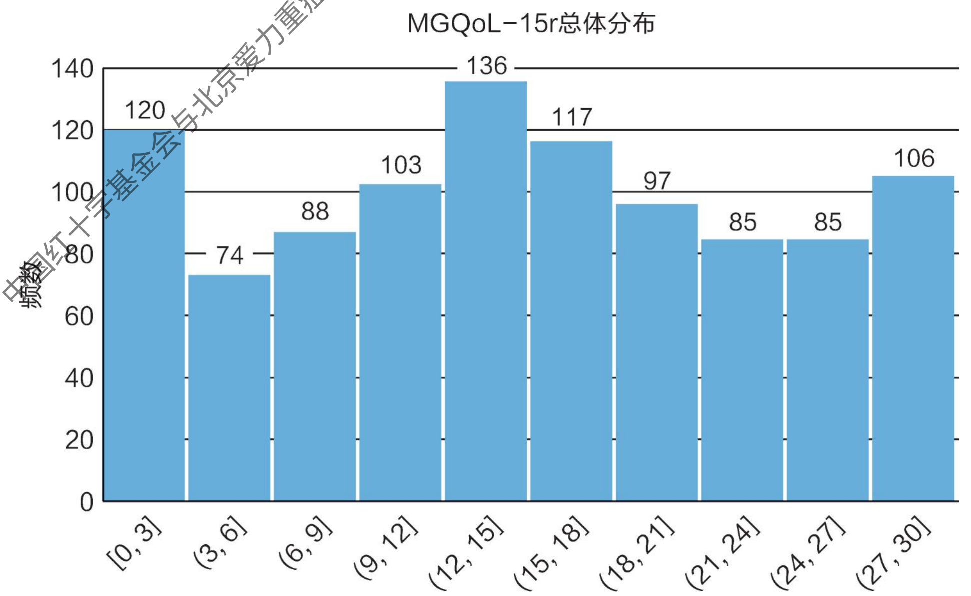
每年新药最高支付意愿在 10 万元以下的患者照顾者中，具体支付意愿的范围在 200-6 万元之间，平均值为 15045.7，中位数为 1 万元。

分类	平均值	中位数	最小值	最大值
患者（N＝879）	12204.5	10，000	0	80，000
患者照顾者（N＝70）	15045.7	10，000	200	60，000

13. 生活质量，疾病影响和社会支持

1）重症肌无力生活质量量表（MGQoL-15r）

本次调研运用了特异性量表 15 条目重症肌无力生存质量量表（MGQoL-15r）评估了成年患者的生存质量。量表测量了患者在移动能力（9 个条目）、症状（3 个条目）、心理健康（2 个条目）和总体满意度（1 个条目）的生活质量，量表得分范围为 0-30 分，得分越高，表明患者生存质量越差。



结果显示，在 1011 名成年受访者中，MGQoL-15r 得分范围为 0-30 分，平均值为 15.2 分，中位数为 15 分。 男性患者的平均得分（15.6 分）略高于女性患者（15.1 分），提示男性患者生活质量略低于女性患者。

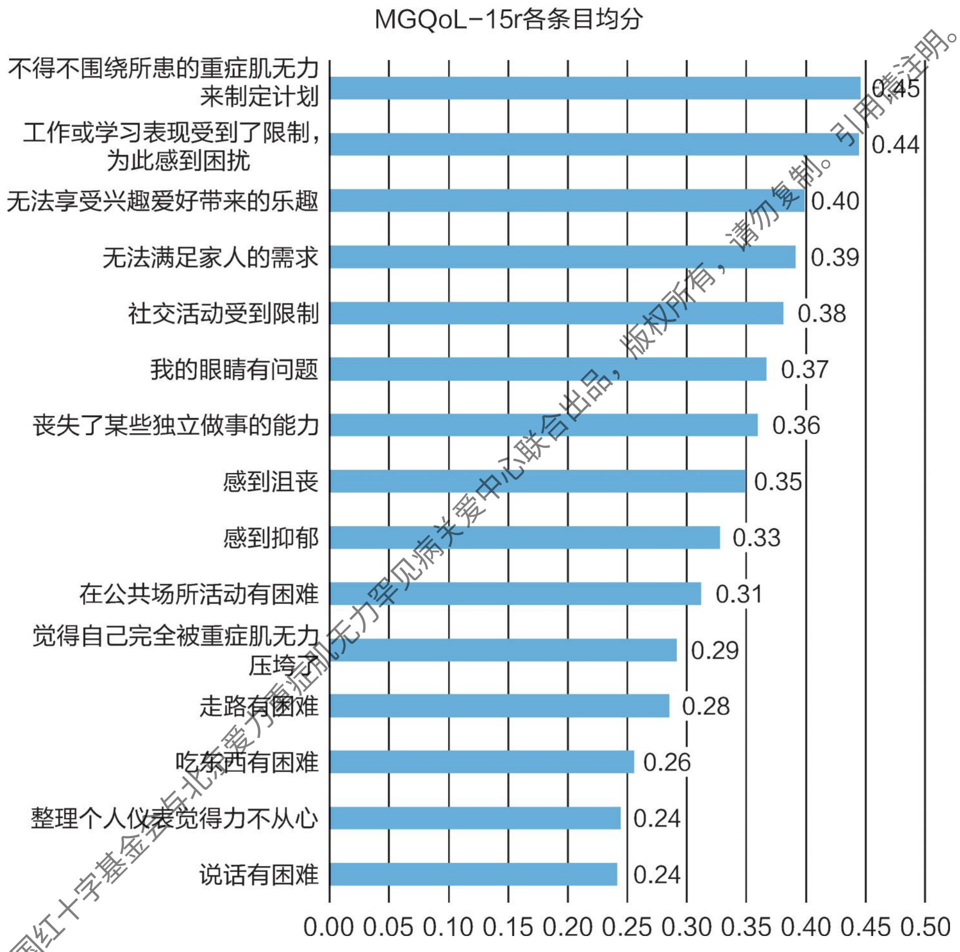
性别	平均值	中位数	最小值	最大值
男性	15.6	15	0	30
女性	15.1	15	0	30
总体	15.2	15	0	30

作为在某种程度上的参考，此次 MG 患者整体反映的生活质量得分低于参与《2018 年中国重症肌无力患者生存状况调研》的患者得分，也更是低于《2020 年中国罕见病综合社会调研》中 MG 患者的得分。 这意味着参与此次调研的患者整体生活质量有所提高。

调研年份	平均值	中位数	最小值	最大值
2018 年	15.9	16	0	30
2019 年	18.4	19	0	30
2022 年	15.2	15	0	30

在此次调研的 MGQOL-15r 各条目中，平均得分最高的为“我不得不围绕所患的重症肌无力来制定计划”（0.45 分）以及 “因为重症肌无力，我的工作或学习表现受到了限制，为此我感到困扰”（0.44 分），代表患者在这两个方面所受到的影响最大。

平均得分最低的条目为“因为重症肌无力，我说话有困难”（0.24 分）以及“整理个人仪态觉得力不从心”（0.24 分），代表患者在这两个方面所受到的影响最小。

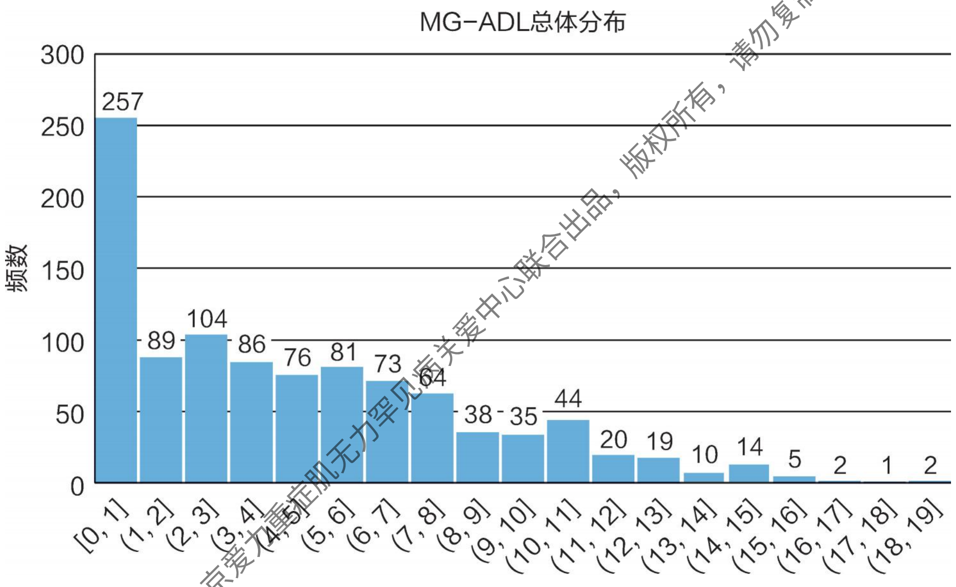


2）自我日常生活能力评价（MG-ADL）

本次调研运用了日常生活能力评分表（MG-ADL）评估了患者的生活能力。 量表评估了患者在说话、咀嚼、吞咽、呼吸、眼睑下垂等 8 项日常生

活的能力情况，量表得分范围为 0-24 分，得分越高，表明患者生活能力越差。

结果显示，在 1020 名受访者中，MG-ADL 分值范围为 0-19 分（量表最高分为 24 分），平均值为 4.9 分，中位数为 4 分。 男性患者平均得分（5.3 分）高于女性患者（4.8 分），提示男性患者的生活能力情况相较于女性患者较差。



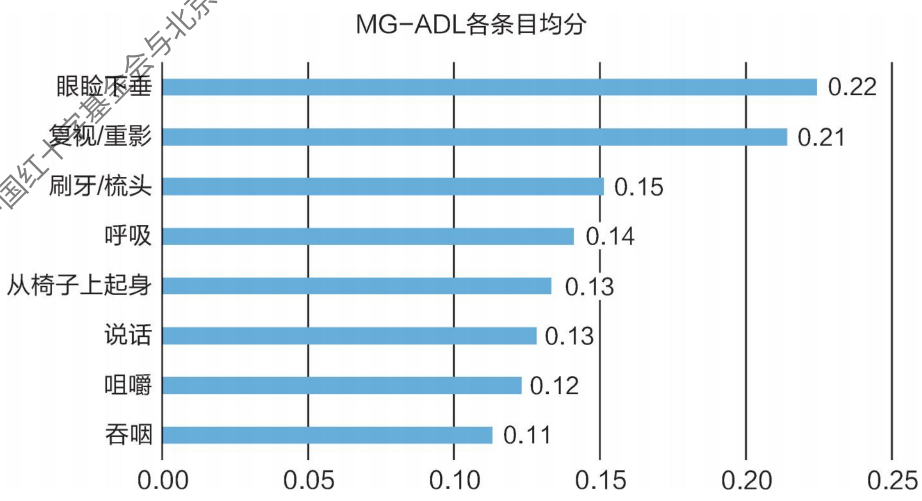
性别	平均值	中位数	最小值	最大值
男性	5.3	5	0	19
女性	4.8	4	0	19
总体	4.9	4	0	19

虽然只能在某种程度上作为一种参考，此次 MG 患者整体反映的日常生活能力评价得分低于参与《2018 年中国重症肌无力患者生存状况调研》的 MG 患者得分，也更是低于《2020 年中国罕见病综合社会调研》中 MG 患者的得分。 这意味着参与此次调研的患者整体日常生活能力相对较好。

调研年份	平均值	中位数	最小值	最大值
2018 年	6.4	6	0	24
2019 年	6.2	6	0	22
2022 年	4.9	4	0	19

在此次调研的 MG-ADL 各条目中，平均得分最高的为“眼睑下垂”（0.22 分）及 “复视/重影”（0.21 分），代表患者在这两个方面所受到的影响最大。

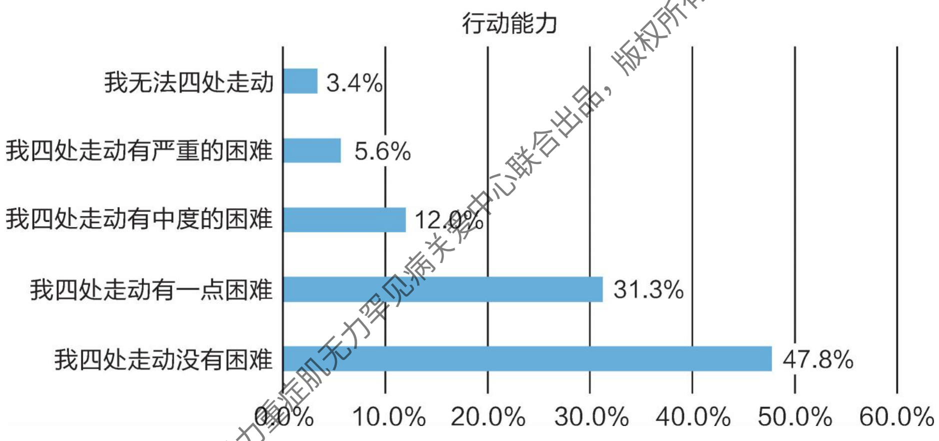
平均得分最低的条目为“吞咽”（0.11 分）以及“咀嚼”（0.12 分），代表患者在这两个方面所受到的影响最小。

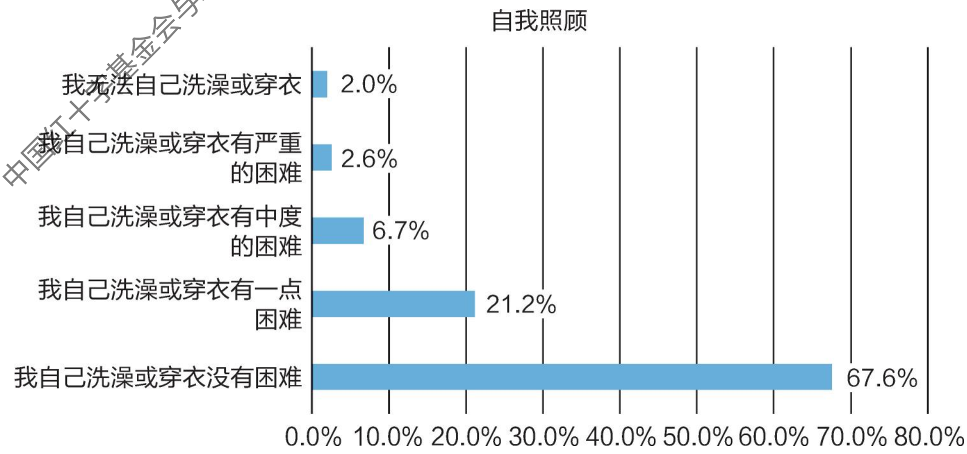


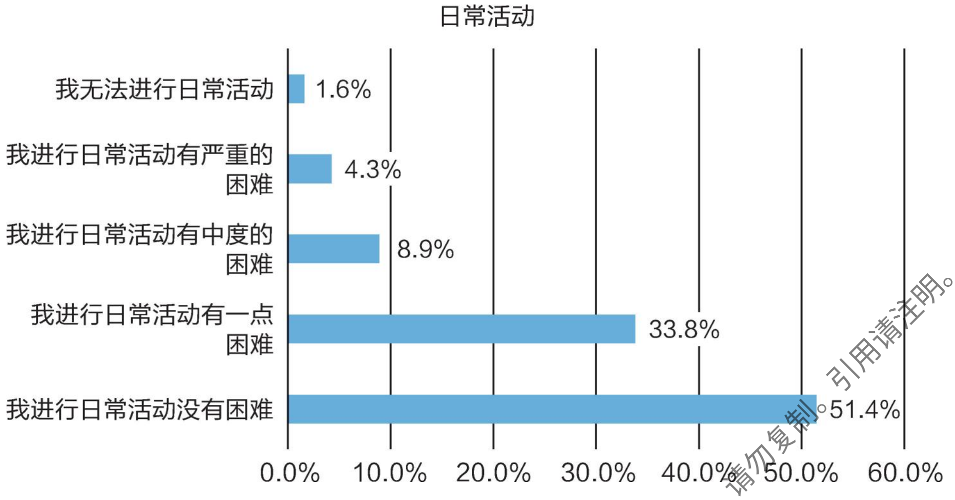
3）整体生活质量- EQ5D

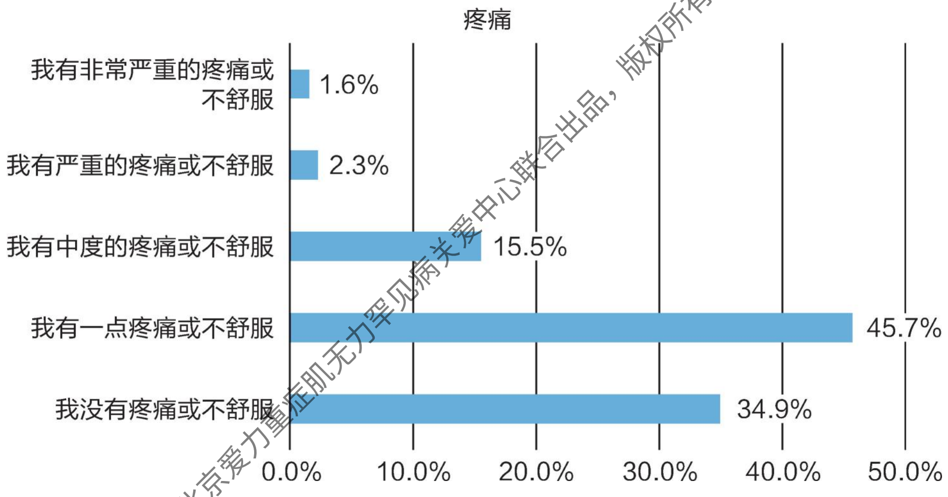
本次调研运用了 EQ-5D-5L 量表评估了 13 岁及以上患者的整体生活质量，涵盖了行动能力、自我照顾、日常活动、疼痛/不舒服和焦虑/沮丧五个维度的生活质量得分。

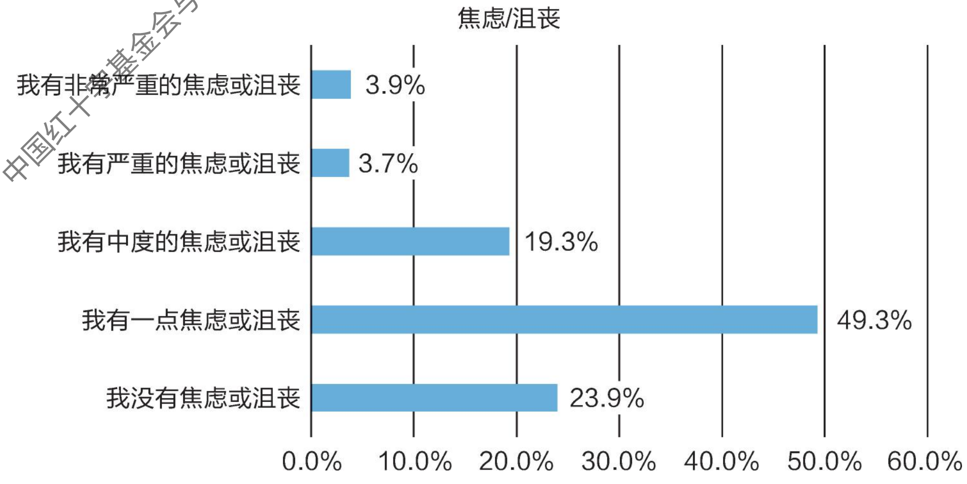
结果显示，在 1011 名患者中，约 50％的患者在行动能力、自我照顾和日常活动方面没有困难；而在疼痛/不舒服和焦虑/沮丧方面，大约 50％患者有一点困难。





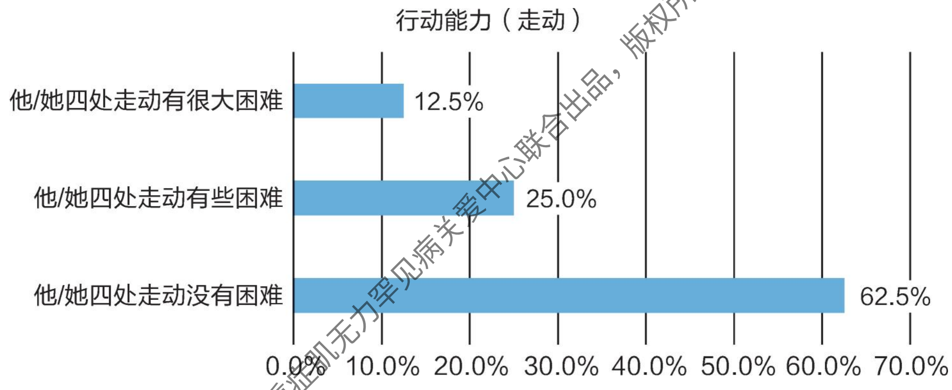

日常活动
我无法进行日常活动
1.6%
我进行日常活动有严重的困难
4.3%
我进行日常活动有中度的困难
8.9%
我进行日常活动有一点困难
33.8%
我进行日常活动没有困难
51.4%
0.0%
10.0%
20.0%
30.0%
40.0%
50.0%
60.0%

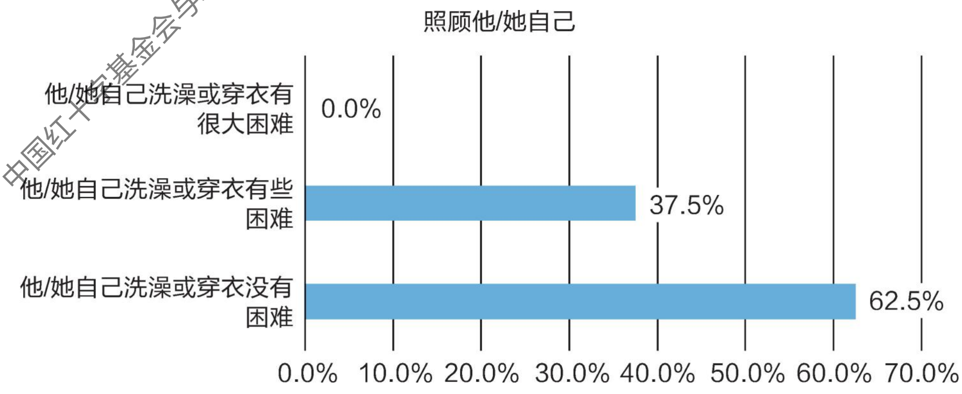

疼痛
我有非常严重的疼痛或不舒服
1.6%
我有严重的疼痛或不舒服
2.3%
我有中度的疼痛或不舒服
15.5%
我有一点疼痛或不舒服
45.7%
我没有疼痛或不舒服
34.9%
0.0%
10.0%
20.0%
30.0%
40.0%
50.0%

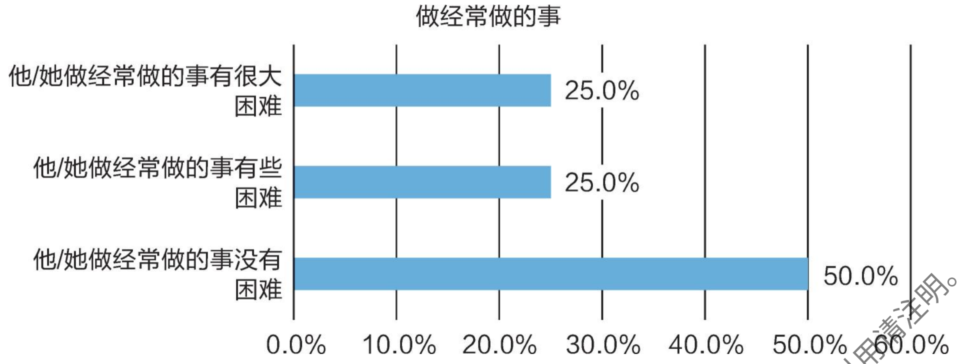

焦虑/沮丧
我有非常严重的焦虑或沮丧
3.9%
我有严重的焦虑或沮丧
3.7%
我有中度的焦虑或沮丧
19.3%
我有一点焦虑或沮丧
49.3%
我没有焦虑或沮丧
23.9%
0.0%
10.0%
20.0%
30.0%
40.0%
50.0%
60.0%

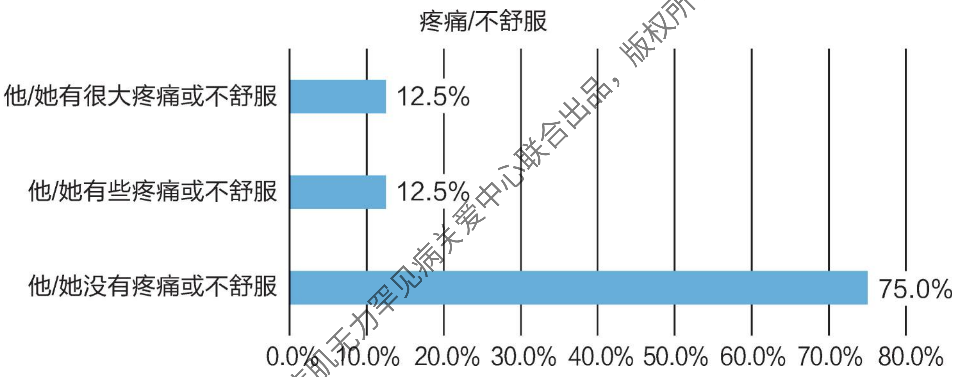
本次调研还运用了 EQ-5D-Y 评估了 5-12 岁患者的整体生活质量，主要涵盖了行动能力（走动）、照顾他/她自己、做经常做的事（比如：上学、兴趣与活动、运动、玩耍、和家人或朋友一起做事）、疼痛或不舒服（比如头疼或者身上痒）和感到担心、伤心或不高兴五个维度。

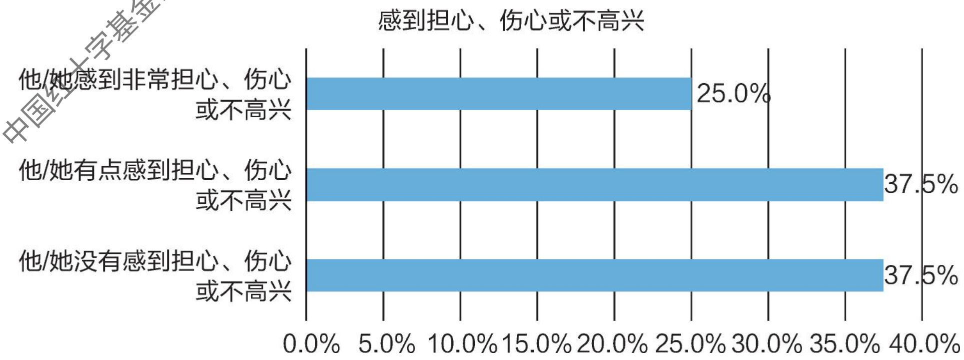
在 8 名儿童患者中，12.5% 的患者在行动能力有很大困难，25% 的患者在做经常做的事方面有很大困难，25% 的患者感到非常担心、伤心或不高兴；而在照顾自己和疼痛或不舒服方面遇到的困难较小，没有患者在照顾自己方便有很大困难，75% 的患者没有疼痛或不舒服，






做经常做的事
他/她做经常做的事有很大困难
他/她做经常做的事有些困难
他/她做经常做的事没有困难
25.0%
25.0%
50.0%
0.0%
10.0%
20.0%
30.0%
40.0%
50.0%
60.0%

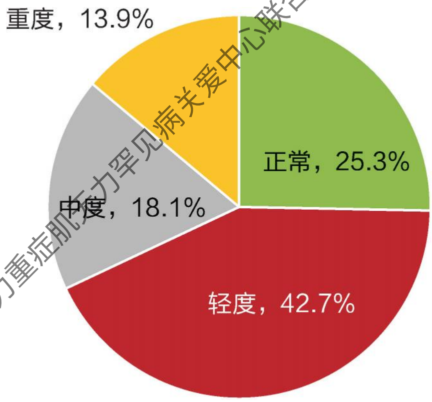

疼痛/不舒服
他/她有很大疼痛或不舒服
他/她有些疼痛或不舒服
他/她没有疼痛或不舒服
12.5%
12.5%
75.0%
0.0%
10.0%
20.0%
30.0%
40.0%
50.0%
60.0%
70.0%
80.0%


感到担心、伤心或不高兴
他/她感到非常担心、伤心或不高兴
他/她有点感到担心、伤心或不高兴
他/她没有感到担心、伤心或不高兴
25.0%
37.5%
37.5%
0.0%
5.0%
10.0%
15.0%
20.0%
25.0%
30.0%
35.0%
40.0%

4）超简焦虑抑郁筛查量表（PHQ-4）

本次调研运用了超简焦虑抑郁筛查量表（又称 4 条目患者健康问卷，PHQ-4）评估了成年患者的抑郁、焦虑水平。 量表包含了焦虑（2 条目）、抑郁（2 条目）两个部分。 量表得分范围为 0-12 分，得分越高，表明焦虑抑郁水平越高。

结果显示，在 1011 名成年受访者中，PHQ-4 得分范围为 0-12 分，平均值为 4.7 分，中位数为 4 分。 男性患者的平均得分（5.2 分）高于女性患者（4.5 分）分，提示男性患者的焦虑抑郁水平高于女性患者。 总体上，重度焦虑抑郁（9-12 分）的患者有 140 人，占比 13.9%；中度焦虑抑郁（6-8 分）的患者有 183 人，占比 18.1%；轻度焦虑抑郁（3-5 分）的患者有 432 人，占比 42.7%；256 名患者不存在焦虑或抑郁（0-2 分），占 25.3%。

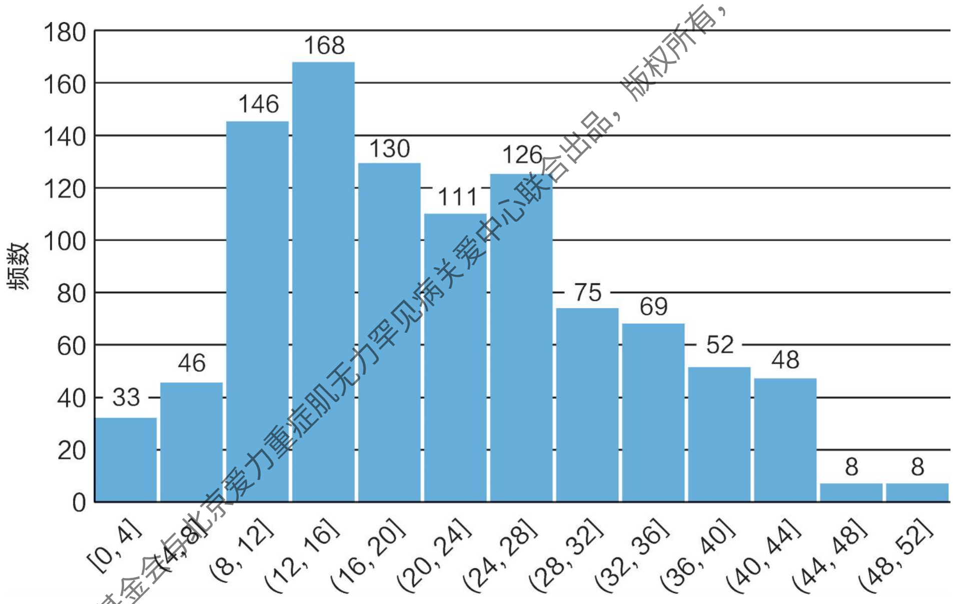


性别	平均值	中位数	最小值	最大值
男性	5.2	4	0	12
女性	4.5	4	0	0
总体	4.7	4	0	12

5）慢性病治疗功能评估 - 疲劳量表（FACIT）

本次调研运用了慢性病治疗功能评估- 疲劳量表（FACIT）评估了患者的疲劳程度。量表包含了13个条目，评估过去七天内疲劳对患者生理、功能、心理及社交等方面的影响。量表得分范围为0-52分，得分越高，表明患者的疲劳程度越轻。

结果显示，在1020名成年受访者中，FACIT得分范围为0-52分，平均值为21.6分，中位数为20分。男性患者的平均得分（22.8）高于女性患者（21.1），提示女性患者过去七天疲劳程度相对男性更严重。

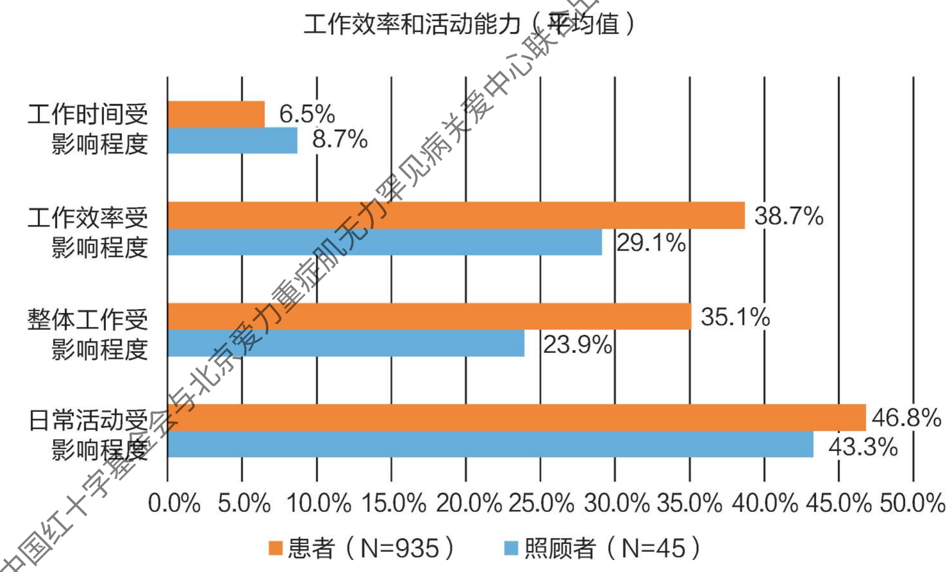


性别	平均值	中位数	最小值	最大值
男性	22.8	22	0	52
女性	21.1	20	0	52
总体	21.6	20	0	52

6）工作效率和活动能力（WPAI）：患者 vs 照顾者

本次调研运用 WPAI 量表调查了目前工作的成人患者和患者照顾者受健康问题或照顾患者的影响程度百分比，即有多大百分比的工作和日常活动受到了影响。

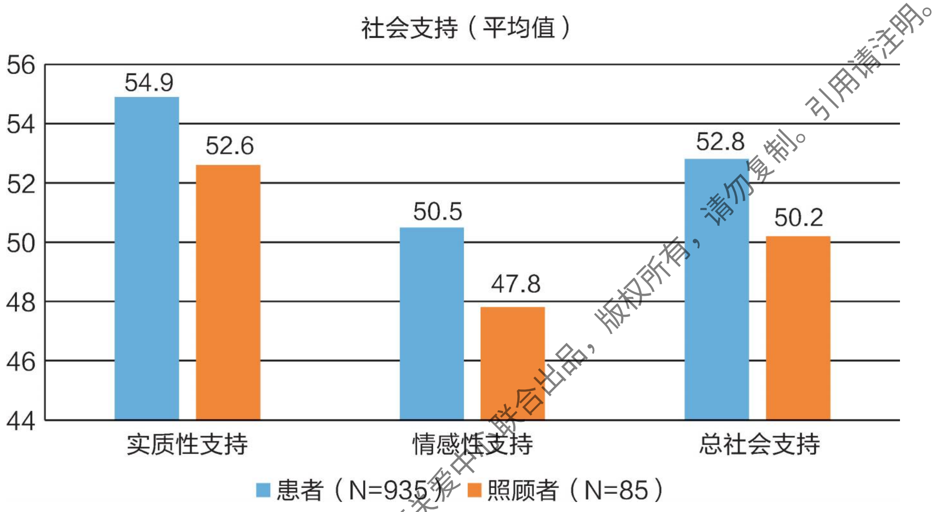
在 298 名目前工作的患者中，在过去七天由于健康问题平均缺勤 3.6 小时；在 45 名目前工作的患者照顾者中，在过去七天由于健康问题平均缺勤 6.2 小时。 在工作效率、整体工作和日常活动方面，成年人患者在日常活动方面受影响程度高于患者照顾者；在工作时间方面，患者照顾者的受影响程度百分比高于成人患者。



7）社会支持（mMOS-SS）：患者 vs 照顾者

本次调研运用了 8 项 mMOS-SS 量表评估了成人患者整日和患者照顾者整体接受社会支持的情况，主要调查了实质性支持和情感性支持两个方面，总分在 0-100 之间，分数越高，代表社会支持水平越高。

总体而言，患者整体的社会支持平均得分（52.8 分）高于照顾者整体平均得分（50.2）。 患者在实质性支持和情感性支持两个方面的得分均高于患者照顾者，分别为 54.9 和 50.5；而患者照顾者在两方面受到的社会支持平均得分分别为 52.6 和 47.8。



第三部分
重症肌无力患者病患旅程

14. 病患旅程调研简介

1）病患旅程各阶段简介

该重症肌无力患者病患旅程描述了患者从发病到病情稳定过程中所经历的求医、治疗和康复的历程。通过 33 例患者和家属访谈，以及 7 例医生访谈，该报告将重症肌无力患者病患旅程分为以下七个阶段。

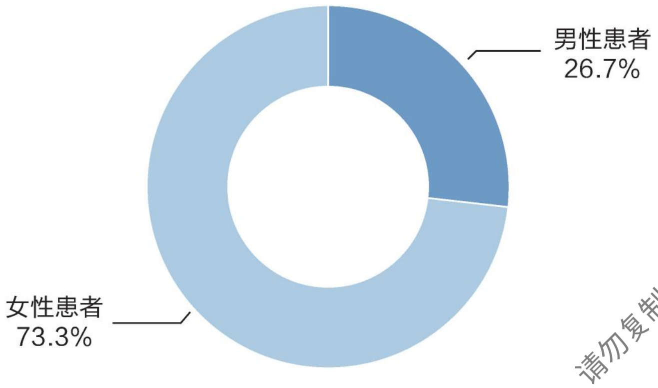
病患旅程阶段简介

病患旅程阶段	说明
发病	发病起始症状，病患对该症状的理解及采取的求医行动
确诊	病患的确诊历程，包括病患首次就医的科室，在确诊前辗转过的科室，最终确诊所用方式，及确诊关键因素

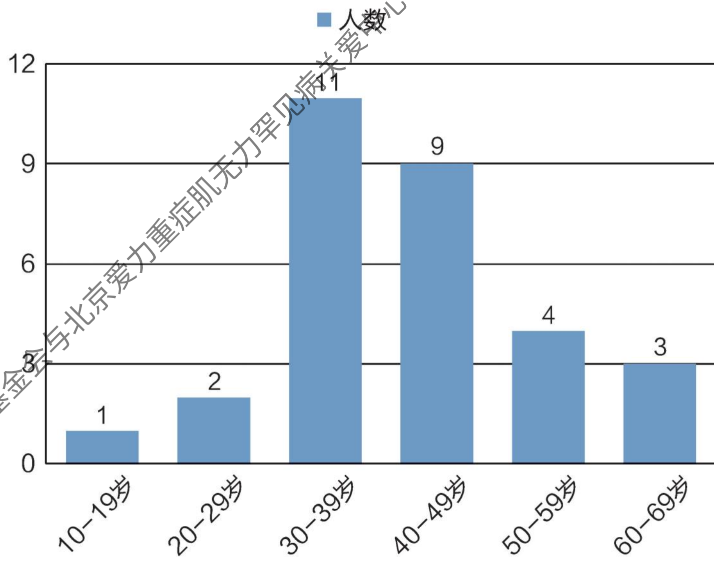
病患旅程阶段	说明
治疗	病患所使用的治疗手段，包括服用一二三线药物的情况及其副作用，实施胸腺切除手术及术后效果，以及采取的其他治疗方式如丙球冲击、血浆置换、中医治疗等
复发与危象	病患在治疗过程中所经历的复发与危象情况，以及其原因和处理手段
病友组织支持	病患在治疗过程中受到的病友组织的药物、资金及其他社会支持
康复	病患当前所用的康复措施及康复需求
当前病情及其他需求	病患当前的疾病状况，及其在心理、社会保障、就业等方面的其他需求

2）患者基本人口学特征

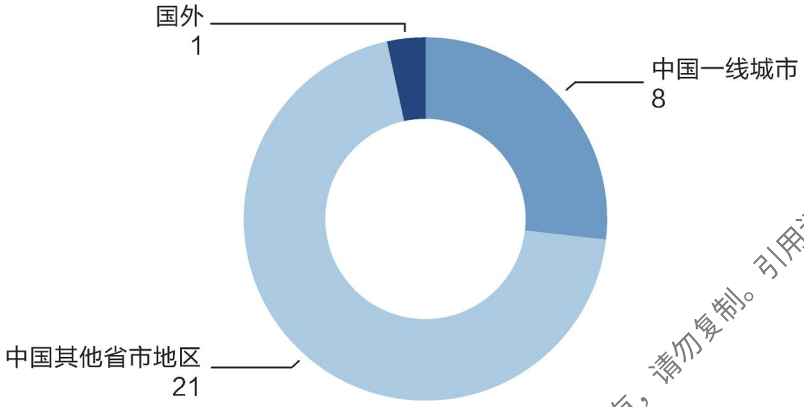
在本研究所收集到的 30 位病患信息中，有 22 位患者为女性，8 位患者为男性。 患者年龄在 18 至 65 岁之间。 有 8 位患者居住于北京、上海、广州、长沙、成都、杭州等一线城市，有 21 位患者居住于中国其他省市，另有 1 位患者居住于国外。 所有患者全部为全身型患者，约 70% 的患者进行了胸腺切除。 此外，本研究中有 86.7% 的访谈病患能够被包括城镇职工、城镇居民、新农合、商业保险或其他保险等至少一种医保类型所覆盖，但仍有 13.3% 的访谈病患没有医保覆盖，需要自己承担全部医疗费用。



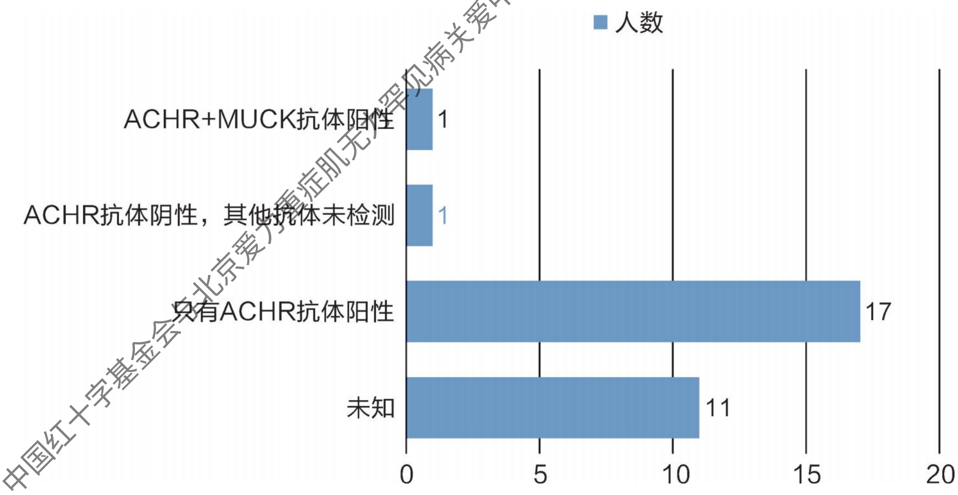
病患性别分布



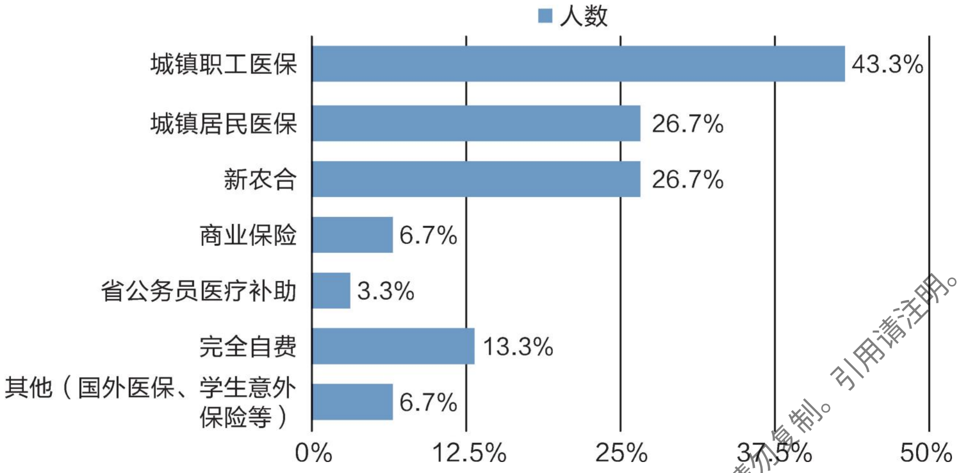
病患年龄分布



病患居住地分布



病患抗体类型分布



病患医保覆盖情况分布（共 30 人）

3）病患、家属及医生访谈分布

患者及家属访谈分布①

	患者访谈				家属访谈		
	一线治疗	一二线徘徊	二线治疗	难治	一线治疗	二线治疗	难治
北京/上海/广州/长沙/成都/杭州	2	-	3	3	1 *	1 *	1 *
其他省市地方及国外	7	2	4	5	1	2	1

---

① 注：家属访谈里，标 * 号的家属为本研究访谈过的病患的家属，不标 * 号的只访谈过家属，没有访谈过病友本人

医生访谈访谈分布

	神经内科	胸外科	中医
北京/上海/ 广州/长沙/ 成都/杭州	2	1	1
其他省市及地方	1	1	1

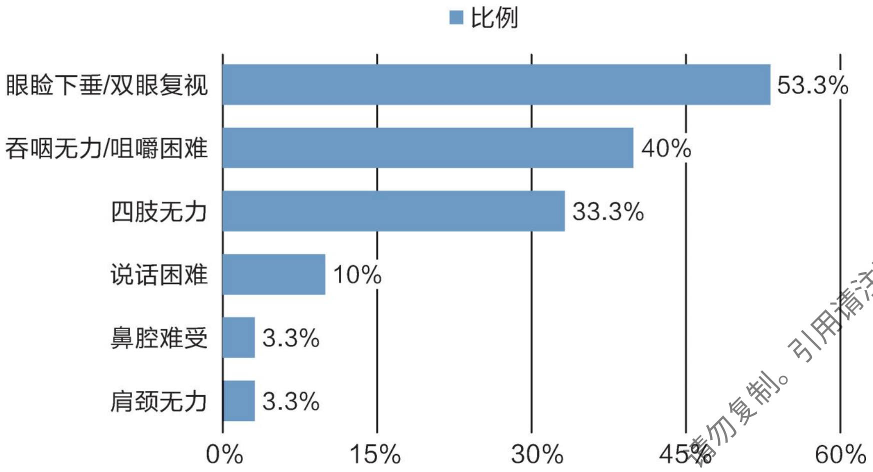
15. 病患旅程

1）发病

根据《中国重症肌无力诊断和治疗指南（2020 版）》，重症肌无力在各个年龄阶段均有可能发病。在本研究受访的重症肌无力患者中，患者首次发病的年龄最早为 6 岁，最晚为 55 岁。本章节将介绍患者在首次发病时的主要症状及相关理解和行为。

1.1 症状

重症肌无力患者的首次发病症状不一，其中眼睑下垂或双眼复视为访谈患者中最常见的首发症状，其他症状包括咀嚼无力、吞咽困难、发声困难、行走或抬臂无力、上楼梯困难等，患者具体首发症状分布如下图所示。



患者首发症状分布（共 30 人）

值得注意的是，有约三分之一的患者在首次发病后症状迅速进展，可由单一症状发展成全身型，或甚至累及呼吸肌。

“最开始时咀嚼无力，戴牙套很累，然后经常觉得疲劳，还有是那个头发梳头，两个手都抬不起来。 然后就没办法就把头发剪短了。 再后来是眼睛老是抬不起来，一会儿它就耷拉下来。 还有就是发音，就是说是就是老师功课也特别多，一下留下好多功课要背那个书。 然后背了好多书。 时间长的话。 就是就说不出声音来了。”

——P-XJ-03

“我其实发病没多长时间，横竖到不了十天半个月，我发病很快，等我到医院的时候，四肢都已经没什么力气了。”

——P-BJ-09

1.2 认知

大部分重症肌无力患者及其家属在患者首次发病时都未引起足够重视，这部分是因为关于罕见病的知识尚未在公众层面得到很好普及，很多人

从未听说过重症肌无力这种疾病；部分是因为患者及其家属尚未建立起成熟的现代求医意识，反将重症肌无力的症状归因于“邪气”、癔症等迷信元素，或归咎于精神问题。 例如，有很多病患在正式前往医院检查之前，会先求助于当地“大神”，或求助于无正规行医资格的小诊所，导致病情延误。

“（那些大神）就会给你整一些什么偏方，类似于取药什么的，再烧点纸，写个符给你，烧完了之后喝水。 我也喝过的。 完了还要破关，也弄完了，好像在你们那儿没有听过，在我们这儿有，要闯什么关。 还有好多这类的，有跳大神的，还有供佛的，哎呀，反正，那时候在不断地折腾，到最后越来越严重。”

——P-LN-01

另有一些病人虽能感到明显的肌肉无力症状，但却被自己或家属反复轻视，认为仅仅是因为疲劳或抑郁所致，甚至有些家属认为是病人自己疑病过甚。

“因为当时时好时坏的，今天不好了，等我休息了一天以后，后面就又好了。 我也没想到往（重症肌无力）这方面去考虑，所以也没想到去看。 但后面就不行了，后面完全是每天这样了，所以我去看，当时看了以后，他（患者丈夫）认为我是胡思乱想，当我抑郁症，给我差不多吃了一年的抑郁症的药，还是没效果。”

——P-ZJ-24

有少部分病人能在发病后很快意识到自己得了重症肌无力，这多半得归功于病人自己的健康素养或网络素养。 例如，有的病人表示因为在初中生物课上学习到过自身免疫疾病的相关知识，或在电视节目中看到过重症

肌无力病人，所以能在发病后推测出自己肌肉无力的原因；另外，有多位病人表示他们在发病后通过互联网搜索相应症状，猜测出自己可能的病症，从而能及时求医。

“我记得很清楚，是 12 月份出现的，就是手开始有点麻木，然后一直到了二月份，越来越严重，就是突然有一天左眼的眼皮就掉下来了。 然后说话我就觉得很费劲。 就两个月多月的时间。 然后我就自己百度，然后就发现说是重症肌无力，然后我就直接到湖南某省级三甲医院的神经内科去确诊。”

——P-HN-18

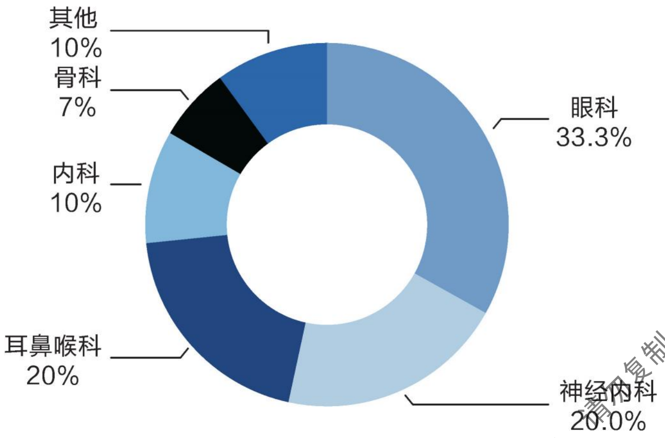
1.3　行为

由于对最初重症肌无力发病症状的理解不同，患者在发病后的求医行为上也有很大差异。 有些病患能在自己或家人认识到疾病的严重性后及时前往当地医院求医，有些因为病情不断波动起伏，所以在发病后会花较长的时间等待或观望病情的发展。 另有一些病人虽然有求医的意识，但因为现代医疗知识的缺乏，而优先选择向无正规资格的门诊或民间偏方求助，这大概率导致延误治疗，从而引起症状加重。

2）确诊

2.1　首次就医科室

由于重症肌无力患者的首发症状不一，所以病患的首次就医科室也有很大差别。 因为眼肌无力是重症肌无力患者的最常见症状，所以有三分之一的患者首次去眼科求医。 其他病患首次就医科室包括耳鼻喉科、神经内科、内科、骨科等。



患者首次就医科室分布

尽管有约五分之一的患者能在首次就医时就准确选择神经内科，但这并不代表他们能一次确诊。 因为中国各省市地区医疗资源水平的不均衡，仍有许多神经内科的医生也不了解重症肌无力这种疾病，反而将病人误诊为其他病症。

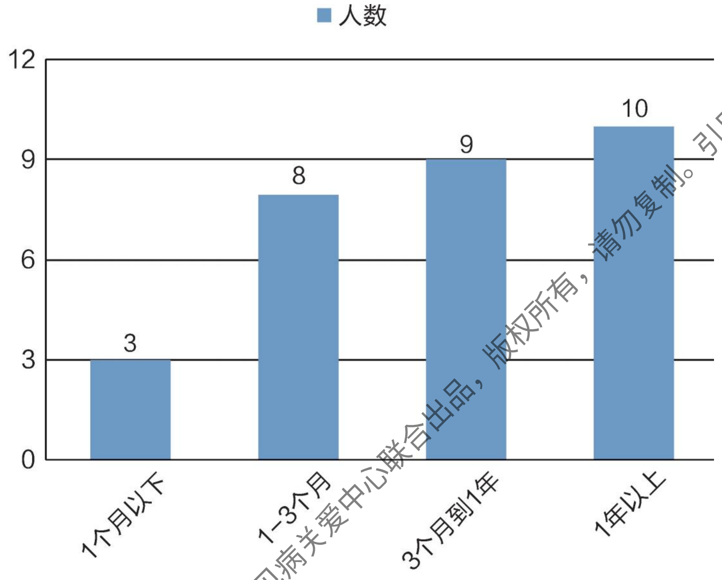
“最开始是去的郑州的＊＊＊医院，挂了个神经内科，因为我学的生物课本上有一个介绍说，那个胸腺的神经传导问题，可能会导致这种无力现象。 然后我自己给大人提醒，让他们带我去的神经内科。（到了那边之后）医生就是尝试了以下，让我做了几个动作之类的，也没有怎么检查，就拿了一些葡萄糖酸　之类的营养补充剂给我。”

——P-XJ-03

2.2　辗转科室

在本研究访谈的重症肌无力患者中，仅有 16.7% 的患者能在一次就医后得到确诊，另外 83.3% 的患者都在多次辗转其他科室后，才能来到神经内

科得到确诊。确诊对出于农村和欠发达地区的患者尤其困难，大部分患者在首次发病后，都要经历至少一个月的时间才能得到确诊，甚至有三分之一的患者从发病到确诊时间长达一年。



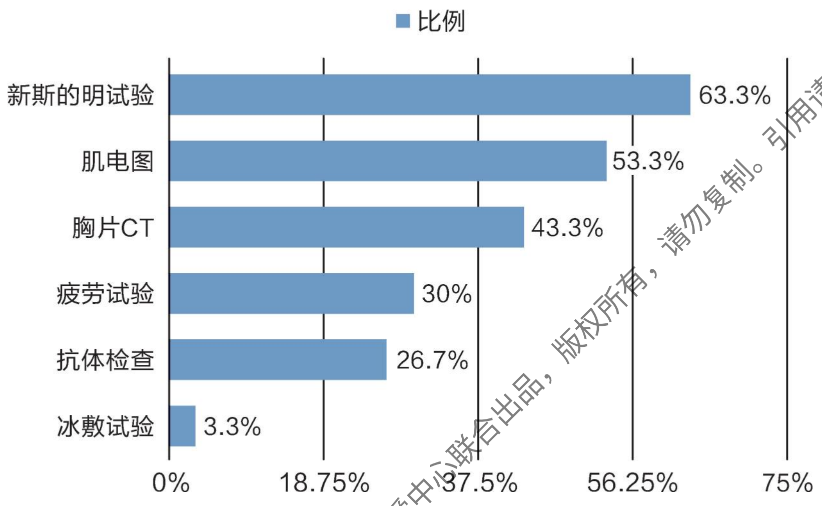
患者从发病到确诊所用时长分布

值得注意的是，有些关键因素有利于患者及时快速得到确诊，这些关键因素包括：能够被经验丰富的眼科或耳鼻喉科医生转到神经内科；病人能通过网络搜索症状（或通过电视剧、书本等途径）了解重症肌无力，因此去神经内科看诊；病患有熟人为神经内科或相关科室医生，推荐去神内看诊；或能在医院导诊台准确被导诊去神经内科等。而对于未能得到及时确诊的病患，常见的确诊障碍包括：当地医疗资源水平有限，在眼科、耳鼻喉科、内科等其他科室没有被及时转诊；病患或家属未能引起足够重视，或先前往非正规推拿诊所等导致耽误确诊。

2.3 确诊方式

重症肌无力患者得到确诊的方式不一，三分之二患者的确诊方式均包

括新斯的明试验，即通过肌肉注射甲硫酸新斯的明试剂后观察肌力改善的方式判定试验结果为阴性或阳性。 其余确诊方式包括肌电图、血清抗体检查、胸片检查、疲劳试验等。



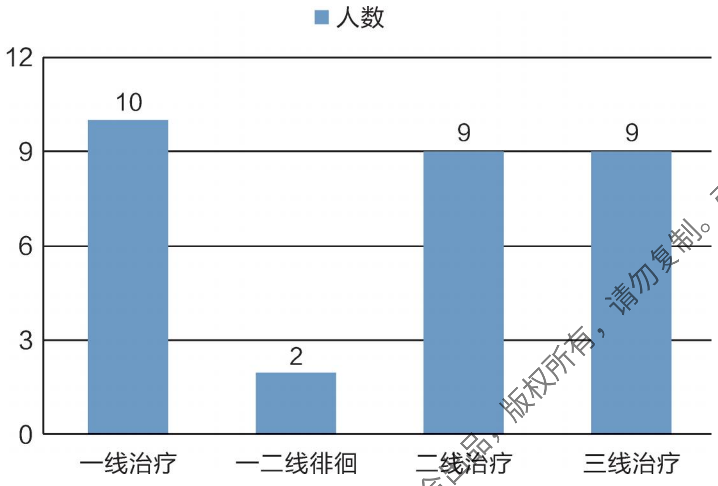
患者确诊方式分布

3）治疗

3.1 药物

重症肌无力的药物治疗分为一线治疗、二线治疗和三线治疗三类。 常见一线治疗的药物包括胆碱酯酶抑制剂（溴吡斯的明）、激素、硫唑嘌呤（AZA），其中溴吡斯的明和激素为本研究受访病患中最常用的药物。 常见二线治疗药物包括霉酚酸（MMF），环孢菌素（环孢素）和他克莫司。 常见三线治疗药物包括环磷酰胺、甲氨蝶呤、利妥昔单抗、依库珠单抗、生物靶向药等。 本研究中采用过单抗或生物靶向药治疗的患者，全部通过参与新药开发临床试验接触到此类药物。 有些临床试验为患者的主治医生所推荐，

有些是患者从病友组织或相关公众号了解到相关实验信息。 本研究受访病友中主要采用一线、二线及三线治疗的病患比例分布如下图所示。



患者一二三线治疗分布

患者的用药效果与多种因素相关，包括患者本身的抗体类型、患者是否为“难治型”患者，患者本人用药的依从性以及与医生的沟通程度。其中，一些受访患者并未能遵医嘱用药，或在未与医生妥善沟通的情况下随意加药、减药、换药，这也是导致患者药效不佳、病情反复的一大主要原因。

“因为家里那个弟弟妹妹说，这个激素吃多了不行，因为这些药有副作用。 我就把它停了，也没问医生，还问啥医生？ 我内心已经放弃希望了。然后症状就越来越重。”

——P-LN-06

值得注意的是，患者的依从性除与个人健康素养和医疗知识水平相关外，也与其他结构性因素相关，如许多患者因为家庭经济状况不佳而长期需

要在外打工，所以无法定期看同一个主治医生，从而导致病情复发。 如患者 P-NMG-07 上完初中后就去外地打工，打工时首次出现症状。 在经过确诊后，又跟随亲人从内蒙古前往河南打工，此后又回内蒙古包头，辗转包头、呼和浩特多地。 每到一处，该患者都在当地寻找医生重新制定治疗方案，这也导致其病情反复多次。

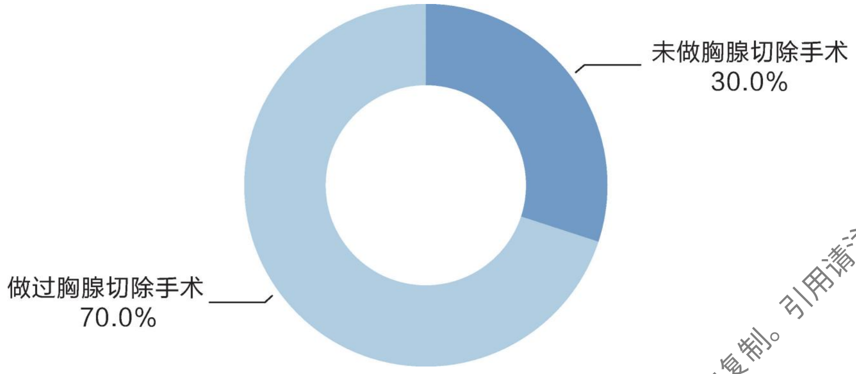
受访患者也提到药物的副作用是他们较为担心的一点。 如有些患者畏惧服用激素，因为激素可能导致体重增加、向心性肥胖、血糖血压升高、骨质疏松、股骨头坏死等副作用。

> *“访谈员：您不想吃激素主要是考虑的什么原因呢?“*
>
> *“患者：吃的时间太长了，整个脸都肿得特别大。”*
>
> *——P-BJ-12*

因为不同地区医疗水平之间有所差异，并不是所有患者都能在服用激素的同时服用钙剂和双磷酸盐类药物减轻相关副作用，所以有患者出现了股骨头坏死的症状，在紧急停用激素后又导致病情复发。 此外，患者还提到溴吡斯的明的副作用包括腹泻、胃痉挛等，硫唑嘌呤可能导致骨髓抑制、肝功损坏等，以及他克莫司的副作用包括白细胞减少等。

3.2 手术

胸腺切除为重症肌无力患者常见的非药物治疗方法之一。 根据《中国重症肌无力诊断和治疗指南(2020 版)》，合并胸腺瘤的重症肌无力患者应尽早进行胸腺瘤切除手术，大部分非胸腺瘤的重症肌无力患者也可通过胸腺切除手术缓解症状。 在本研究采访的重症肌无力患者中，有 70% 的患者进行了胸腺切除手术。 患者胸腺切除的方式包括开胸手术、微创手术及达芬奇系统机器人手术。



患者实施胸腺切除手术比例分布（共 30 人）

胸腺切除手术起效时间不等。大部分患者在胸腺切除手术后表示有明显改善。如一位患者所言：

“我做的是微创手术，感觉就是做胸腺切除手术以后，能比以前强 80% 左右，好了很多很多。我做手术之前就是说说话、走走路都费劲，跟人家唠嗑超过五分钟就吐字不清了。说话口水比较多，最严重的时候两个眼睛完全闭上了，转都转不了。做完之后感觉就很好，像吃仙丹一样，效果特别得快，眼睛当时就睁开了，口水也少了。”

——P-LN-06

但也有患者表示胸腺切除手术对其用处不大，进行手术后，重症肌无力病情并没有明显好转，甚至可能会带来一些副作用，如一位患者所言：

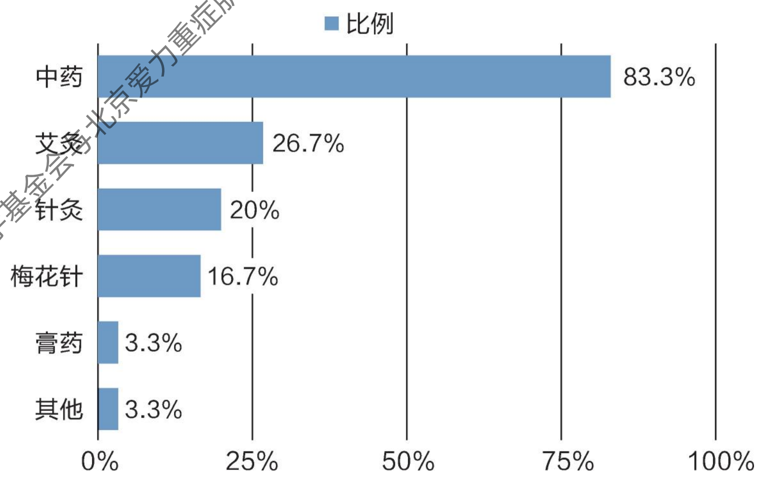
“我胸腺手术完之后还得检查指标，就发现我血小板开始减少。有的病友说这是胸腺手术常见的反应。因为我属于是免疫性血小板减少，也属于免疫性疾病。我就认为这是这个手术导致的。因为人体的这个胸腺不是人体最大的免疫器官吗？把它切除了，导致我这个现象，感觉上我以前是没

有的，因为手术之前我也做了检查。 我手术完之后在那儿观察了大概有半个多月。 他也没有说过这个血小板减少症是怎么引起的。 那大夫就说，我最开始手完术之后血小板很低，隔了三四天之后，血小板又升回来了，但是也没有达到那个正常值。 大夫就觉得好像是手术的应激反应，它会回弹回来，但是它一直没有弹回来。”

——P-LN-01

3.3 中医

重症肌无力患者常采用中西医结合治疗的方式管理自己的病情。 常用中医治疗手段包括服用中药、针灸、梅花针等，也有患者会贴中医膏药等方式治疗疾病。 本研究受访患者中采用中医治疗手段的人数分布如下图所示。 患者对中医的疗效评价不一，有人认为丝毫没有作用，有人认为与西药相辅相成，可以相对减轻西药对自己身体造成的副作用，也有少量患者目前仅靠中药维持病情稳定。



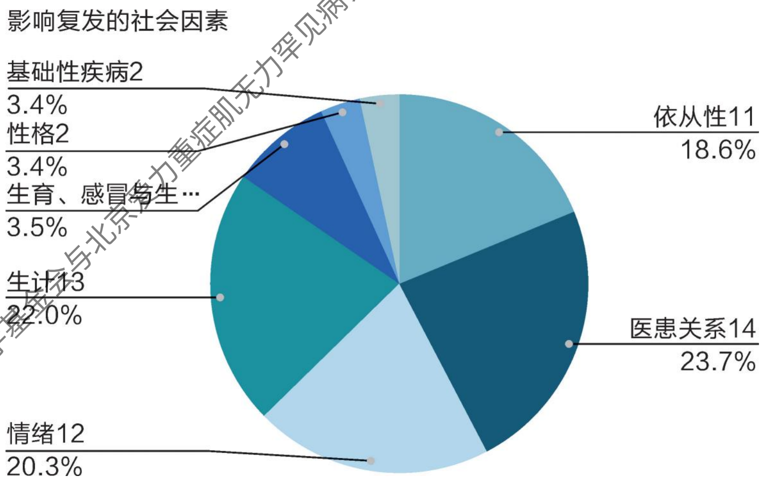
患者采用中医手段治疗人数分布（共 30 人）

4）复发与危象

4.1　复发比例与次数

由于疾病本身的波动性及长期性，重症肌无力患者需要长期严格地通过吃药来控制病情，同时也要遵守相应的生活习惯来控制情绪。 我们访谈的全身型重症肌无力患者中，30 例患者中有 28 人在首次确诊后，重症肌无力都反复发作，甚至经历危象。

我们根据访谈编码的影响复发的社会因素，粗略统计了一下导致患者病情反复的主要因素，分别为：依从性（药物断货、患者自行加减药等）、情绪（病情影响、家庭关系、亲密关系等）、医患关系（无固定的主治医生、不相信西医等）、生计（经济原因、身体劳累、学业负担等）、生育、感冒与生理期、性格、基础性疾病等。



4.2　复发与危象原因

造成复发的原因有很多，总的来说，患者在疾病管理过程中如果没有与医生建立起稳定的信任关系和沟通机制是影响病情的首要因素，药品断货

及副作用等也会导致患者出现复发。 在此基础上，身体劳累、情绪影响、甚至是性格因素通常会成为导致患者病情复发的触发原因。 此外，还有一些患者个人的生理因素，例如感冒受凉等，就女性患者而言，生理期、怀孕、生产、坐月子等也有可能会成为病情复发的触发时间。 以下是一位比较典型的病人的复发原因分析：

P-LN-01，女，20 岁确诊，此后多次复发。

第一次复发因为溴吡斯的明断货；

第二次复发因为吃激素导致股骨头坏死，医生让完全停药，症状立即加重；

第三次复发因为父亲去世，母亲随后又确诊为糖尿病综合征，自己情绪受到冲击；

第四次复发因为在姨妈家的早餐店帮忙，烟熏火燎，呼吸不好，又感到过于劳累，以至于出现危象，被医院下发病危通知书。

患者依从性

最常见的情况是患者没有遵医嘱用药导致病情复发，此外还有在病友互相之间的交流过程中获知其他病友尝试的其他治疗方式及情况，自行决定换药、加药与减药等，也有病友因为医患关系不和从而抗拒见医生复诊导致病情复发的情况。 看起来是患者的依从性问题，然而依从性背后的引发因素也值得我们关注。

P-XJ-03，女，14 岁发病，从新疆来到上海某医院治疗，冲激素导致症状加重，住院三个月未有好转，医护人员态度恶劣，花费三万多元，后出院回家。 后看到河北某医院和河北某莆田系医院的广告，尝试前去询问和治疗后认为是欺骗患者。 此后对医药，尤其是西医，基本失去信心。 目前仅以小明维持，与父母同住，不能完全自理，无法独自出门。

“觉得在上海的那次治疗对自己是一个很大的打击，哪怕以后去北京治

也差不多。”

——*P-XJ-03*

P-BJ-12，AchR＋MuSK 抗体阳性患者，31 岁发病并确诊。因为遵医嘱减激素仍然过快，导致多次复发。并不十分信任医生，因此频繁更换主治医生，并自己根据自己的状况加药减药。症状较为严重时自己决定回山东老家的医院打丙球蛋白，并自己决定丙球用量。

“老家神内科的医生对这个病不了解，他们说你说要让我们怎么治疗那就不用来，你要来输丙球，那就告诉我们输多少，怎么输，我们给你输。”

——*P-BJ-12*

没有固定的主治医生

部分患者通常因为需要跨省流动打工，没有固定的医生或用药方案，通常只是自己通过感受评估是否需要去医院看医生，又处于家庭或经济原因，而更换或减少去看医生的次数。从另一角度而言，也有患者表明因为想看的医生存在挂号难、就医路程远等不得不被动的更换主治医生或减少复诊的次数。

“从县医院到镇医院，那时候是在武汉＊＊医院，看完吃药，它也没什么缓解。后面我就跟我老公到福建这边来打工了，到福州这边我也跑了很多医院，它还是没有，症状还是没有改善。就是老是推来推去的，就觉得我这么严重了，然后好像你们也没有什么解决方案，也不告诉我怎么办，就是把我推到这边，推到那边。”

——*P-FJ-19*

过于劳累

患者发病后，身体与社会功能都面临调适，通常在平稳期患者会选择回归家庭或职场的角色。 但是也通常会由于继续工作，或因家务照料、照顾孩子等过于劳累导致病情复发，从而影响身体健康状况。

“当时我在超市做收银员，需要跟客人交流比较多。 比如说，多少钱这些的交流哈，然后我就讲不出话来，然后很着急，那时候脾气很暴躁。 因为他们听不懂我要说什么，然后我也不知道我是怎么了，然后就很烦躁。后来吃饭的时候我们吃食堂，我就怎么也吃不下去，怎么也吃不下去。 然后就喝点汤，每天都喝点汤，喝汤也会呛到。 然后那个洗衣服，洗着洗着就没力气了。 然后爬楼梯也是，爬着爬着就上不去了，就这样。”

——P-FJ-11

情绪影响

除了劳动强度之外，情绪是影响病情的重要因素。 生活中，患者常常因为家里亲人去世、家人关系不和、经济和心理压力等因素影响情绪导致复发，这在男性和女性患者都比较常见的。

“我那个亲娘舅离世了。 亲舅舅，家里的母亲咋说呢，舅舅得癌症走的，家里的不甘心，就总觉得这么地那么地，就一天到晚就是闹心，闹心，我也跟着闹心，这么复发的，也是胸腺增生严重了。”

——P-LB-06

感冒或女性生理期

对于重症肌无力病人而言，会格外小心避免感冒，因为感冒或者着凉会

让病情变得严重。但同时，如果女性遇到生理期，身体的新陈代谢机制变化可能也会对病情有影响。尤其是是两个因素叠加时，导致病情复发或者引发危象的情况也不少见。

“去年 11 月份左右又来了一次（月经，笔者注），但是量很小很小的，我觉得是要绝经了。反而我觉得我状态是越来越好。因为这个他们说的重症肌无力患者，只要绝经了状态会越来越好。因为每一次来月经的话，重症肌无力都会加重。只要来月经重症肌无力患者都会有加重。”

——P-LN-01

怀孕生产

由于怀孕带来的女性生理变化，使得怀孕、生产、坐月子成为女性重症肌无力患者病情面临的重要挑战之一。虽然相关文献表明，怀孕与生产对病情的影响有可能是平稳、改善或者恶化，但在我们访谈的 20 位女性患者中，7 位是发病前生育的，6 位是发病后生育的。在发病前生育的患者的表述中，其中有 6 位我们认为其发病的触发因素可能与怀孕生产和坐月子带来的身体情况改变有关。对于有生育需求的女性患者而言，孕期管理应格外注意，整个过程需要神经内科医生共同参与。

P-BJ-09，家庭主妇，生产两次，第二次生产方式为顺产转剖腹产。产前确诊，确诊后要二胎。北京的区医院生产，产后三天危象。

“（激素）减到两片半的时候，我就准备要二胎了，当时前几个月还行，等到后几个月的时候就不好了，就特别地不好了。就吃东西的时候特别不好咽，基本上一天就吃鸡蛋羹。等到后两三个月的时候已经加激素了就开始，我把激素都吃到 6 片了。没有好转。累，不想动，也没看医生。因为那阵还没有真正危象过，虽然说那阵做手术的时候，有给我插呼吸机什么

的，但是没有真正。我自己感觉到经历危象没有那个感觉的，没经历过那个，所以还不害怕。生完孩子第三天我就危象了。其实当时应该剖腹产就好了，我觉得就不至于危象，因为真是生比剖耗费的力气要多。”

——P-BJ-09

患者与家庭的互动因素

性格通常指的是对待周围世界的态度，通常由行为反映出来。在疾病管理过程中，性格比较要强的患者较少让家人参与疾病管理也是其中的典型表现，主要原因是认为自己能解决，不愿累及家人。这类患者往往对周围的家人、朋友、同事交代病情的时候避重就轻，并且很少表达自己需要家人参与到疾病管理或者家庭角色的承担，认为自己能够应付，一旦强度过大就容易导致病情复发的情况发生。这类情况是患者由于性格原因主观上避免家人参与自己的病情控制与管理。

此外，在避免家人参与疾病管理的背后，也有家庭关系、家庭系统支持不足等原因。患病给个人与家庭带来一系列的经济负担和心理负担。对于患者而言，如果感知到无法从原生家庭或组成的家庭里获得足够的支持，也往往被迫选择自己承担，避免给其他家庭成员带来影响。

因此，家庭如何在与病友的互动中给予足够的支持，如何有效地支持患者与家庭之间的互动，是值得我们进一步探讨的议题。

“在这个艰难的环境当中成长的吧，我还是足够坚强，没有把我软弱的一面，即使我吞咽不好了，我就把门关起来，我一个人在阳台上我一个人承受，我真是把我最好的状态呈现给家人们，从来没有把我不好的一面呈现给他们。当时什么都没讲，因为当时就是所有的东西就是自己消化了，所以等到我 2015 年危象之前，整个过程我吃什么药他们都不知道的。”

——P-GD-08

“我都是属于能靠自己就靠自己，绝对不会让家人帮忙，就是这种状态，除非是完全就是走路完全必须得扶着，那我没办法，那我就只能扶着。就是能自己动手，或者自己能行动的时候，我只靠自己，就是包括那个时候最难的时候，就是我连头发都搭不起的话，我就会想着靠在沙发上，把手举起来去扎也是可以完成的，只是这个动作会非常难。”

——P-HN-18

5）康复

5.1　康复措施

访谈过程中，绝大多数患者并没有去过医院的康复科门诊或者进行过相应的康复训练。 在我国的语境下，患者们对于西医康复中的物理治疗、作业治疗、言语治疗这些概念很陌生，

对康复的理解主要是传统中医角度下的康复保健措施，例如八段锦、五禽戏、梅花针等。 这得益于病友组织对这些康复保健措施的推广，例如提供线上训练课程、社群打卡、邮寄康复器材等。 康复器材方面，少数有需要的患者家里会配备轮椅、助行器等，但大部分病友则没有配备，如果在复发或者危象期间，则主要通过家人帮忙这种形式来克服。

5.2　康复需求

在探索患者的康复需求时，很多患者提到如果能开发出来针对重症肌无力患者的康复课程或者训练是必要的。 至于具体是什么方面的需要，他们则很难表达。 由于经济原因，大多数病友及家庭的收入主要花费在看病治疗吃药等方面，因此愿意花费在康复方面等支出相对比较少，主要在希望免费或者支出在几十块到一百元不等，而愿意花费的患者主要关注中医传统下的康复保健措施、康复课程针对重症肌无力的有效性、是否会与康复师

有互动、患者之间的互动、康复课程是否能帮助患者养成长期习惯、康复课程的频率和程度等。

“课程一周一次，希望指导一些八段锦的动作，因为自己一个人不能坚持下来，有老师带容易自律。最好免费。”

——*P-HLJ-26*

“希望有人一对一指导，给挑挑毛病，比如针灸怎么做更好一点，或者作息上要注意避免什么。花费几千块钱以内都可以。日常康复训练或者运动治疗有必要，但不是对每个人都有必要，要量力而行。每个月不超过100块，一个月一次，不能太频繁，因为一周一次不一定有时间。”

——*P-HLJ-24*

“希望康复课程有一起上课的病友，边康复边交流。一个月100元以内，因为长远来看消费不了。”

——*P-BJ-17*

“康复课程需要长期，有耐心，把康复训练融入到生活当中点点滴滴，让病友能坚持。付费可以，但好多途径可以得到信息，有的也不一定非要付费。”

——*P-HN-27*

“想学梅花针。希望老师在线沟通，能够监督。一个月不超过100元。”

——*P-FJ-11*

“对线上课程不太感兴趣，线上没有教练给我调整动作，做错了反而可

能有坏处。”

——P-BJ-12

“希望提供瑜伽、调节呼吸这种舒缓的课程。设置成短视频的模式比较好，因为大家生活节奏都很快。一个月一两百块钱的订阅可以接受。”

——P-JS-13

“希望能帮助我找到适合自己身体情况的运动方式，因为我现在除了走路也不知道能做什么。希望学生和老师之间有互动交流。500以内可以接受。”

——P-ZJ-28

“时间不能太长，身体吃不消。按穴位、调整呼吸可以接受。最好免费。”

——P-SD-04

“希望康复训练能促进患者良好生活习惯的养成，放在衣食住行里面，重点是要能长期落实。”

——P-BJ-22

6）当前病情与社会支持

6.1 当前病情与心理

多数患者在访谈的时候病情已经趋向于稳定。然而，正如疾病本身的波动性，患者对于复发与危象也有心理预期。生活自理方面，除少数病友需要家人的照料与支持，无法完全自理之外，大多数病友可以正常生活与出行，只是不能从事劳动强度太大的任务。

在心理方面，患者存在比较普遍的心理负担，由于面对病情带来的身体

变化、家庭角色与社会角色的变化，从而导致患者普遍存在焦虑、愧疚、抑郁等情绪，这会贯穿整个病患旅程。 主要表现确诊前找不到病因带来的焦虑、治疗过程中病情起伏带来的希望与绝望，这其中医生的沟通、自身角色的调整、疾病的负担都会成为触发因素来影响情绪起伏、确诊后家庭照护带来的愧疚。

“特别绝望，那个时候就是回去把家里头，才 25 岁！ 什么都没有，您知道吧，就是家里头安排安排，等着吧，您告诉还有半年，就是哎呀，就是您要说一点儿不难受，这假的。”

——P-LN-06

“也许有很多病友也会像我这样，自我怎么说呢，自我否定，每天都在自己，怎么说呢，就觉得愧疚，有的会憎恨自己，我以前就是，特别恨自己。”

——P-SD-04

6.2　自我应对

身份调适

患病后身体的变化会带来对病友原本的社会角色的冲击。 患病前，他们原本是父亲、母亲、丈夫、妻子、孩子，但重症肌无力会影响其原本的角色和身份的职能履行，由此带来的身份调适是多数患者都需要面临的状况。这种调适不仅是由于身体体力的变化带来的角色受限，还有由“病人”身份带来的无力感冲击了原本作为“母亲/父亲/丈夫/妻子/孩子”可能的幸福感和满足感，会给病友带来尴尬和遗憾。 而这种协调和转变不仅是病友本身作为个体需要面对的情况，还需要家庭其他成员的角色与功能随之改变。

“在我病最重的时候，影响非常大，我就是走路都很困难。 曾经有一次帮女儿去开家长座谈会的时候，那还不是病最严重的时候，属于波动期。我连进会堂大礼堂的那个台阶，我都上不去，就是只有那么一格，但是我看似非常遥远，我都上不去，只能让老师扶我上去。 平常我从外表来看可能是根本就看不出这些症状的，除非是说在某些行动上面会体现出来，他们才会知道。“您是怎么了”，他们就会问。 我就会这么直接跟她说，我说腿没有力，上不了。 但他们也不会聊，就是不知道是什么原因引起的，是什么病，病状或者不会了解这些东西。 很尴尬的那时候，一般那种运动会或者怎么样，就是爸爸出席，爸爸陪，因为我完全做不了运动。”

“如果情况稳定，还是经常性的抱孩子，但是就是我们家里人就会说，你不要抱他，你没有力气就不要抱，就是他们会出于这种关心，就是说尽量不要抱，就这样子，就会提醒。”

——P-HN-18

除了家庭身份调试之外，患者还面临着职业身份的调试问题。 家庭成员往往能对患者家庭身份对调试提供更多对理解与包容，然而患者的职场环境相较而言则存在更多的不确定性。 通常患病之后，患者也会通过对职场环境的评估采取应对策略，例如跟领导沟通调整工作岗位、争取领导或同事的理解、调整工作性质等等。

“访谈员：明白，所以就是说大家可能知道您的身体情况，但是没有给您调整过岗位，但是有可能说您可能干不了特别重体力的活。”

“受访者：对，虽然是没调岗，但是在那个工作当中，大伙儿都会照顾你，知道吧，没有人会说就是我抬 100 斤，你也得抬 100 斤，没有，没有这种情况。 他说你那个适当，你能干啥干啥，大伙儿在一块儿干就完事了，

就是这样。”

——P-LN-06

然而，我们也遗憾的看到这样的调试不尽成功，很多患者尤其是女性与受教育程度较低、病情影响严重的患者面临着失去工作的境遇，也有不少患者从此失去了对继续求职或者再就业的信心，只能在家做家务或者从事零散的工作帮补生计。

“得病对我找工作有一点影响，因为我之前是做珠宝顾问嘛。就是卖金器的，然后现在吃这个药变成这么胖了，去再找工作就有一点困难，然后长得胖，体力也不是很好，我们就只能做电脑客服之类的吗，收入就会减少很多。”

——P-HN-16

生活调整

为了适应病情对身体与生活带来的挑战，患者往往会从衣食住行等各方面调整自己的生活。避免独自出门、调整工作强度、注意不要感冒着凉、选择适合自己的运动及康复方式等，而随之而来的生活圈与交际圈发生的变化。部分积极的患者会主动调整家庭、朋友与同事的交际圈，从而让周围的人理解身体的变化、活动范围与承受强度以便更好的适应。

“打一个比方，大伙儿都能理解的比喻，完事把你的病说出来。完事儿别让人感到压抑，你也别让人感到厌烦知道不，如果你让他感到烦的话，这你就被孤立出来了，你进不到那个群体里去，你就怎么生存呢。得想办法让别人能理解你的方式介绍你自己，你不能把你自己摆一个很高的位置，也不能摆一个很低的位置，你知道不，就是农民市民也好，你就是再怎么样，

你得跟他们摆在一个差不多的位置。完事儿你告诉他，我那个有的时候可能是不如你们，我需要帮助，但是别的时候，我也能帮助你。”

——*P-LN-06*

未来选择

疾病也会影响病友对未来的预期与选择，病情使得患者的人生轨迹从此改变。对未来选择的影响，一方面是影响患者个人的生活，例如学业、求职、择偶等，另一方面影响患者的家庭选择，包括家庭经济规划、角色期待、养育等。疾病无形中与患者未来选择的连接，往往对于患者而言是被动和无奈的，身体健康状况成为选择的优先项，他们及家庭都面临着如何去适应和调适的挑战。

“访谈员：就是以前的排序，可能您以前自己认为就是比如说要去拼事业什么的，去挣钱，或者说去有一个好的职业，但是可能现在来说就是身体会排在最前面对不对?

患者：是的，因为我觉得身体健康才是最重要，其他都是浮云了，特别是我们经历过。工作的目的也是为了有一个好一点的社会保障，也不图赚钱，更重要是扮演好做母亲的角色。”

——*P-HN-18*

6.3 家庭应对

患病后，除了患者个人的要应对病情对生活带来的挑战之外。由于重症肌无力本身的疾病慢性特点及对疾病管理的要求，对于患者的家庭而言，无论是从照护、经济与情感方面都需要对病情予以应对。此时，家庭对疾病的应对方式对患者而言十分重要，积极的应对会对病情十分有帮助，然而

消极对应对则会影响病情的控制与恢复。 因此，这里讨论的并不全都是积极的应对。

照护支持

由于重症肌无力发病时症状发展的渐进性，往往身体功能受限，症状才从视力、吞咽等功能开始，进而影响到四肢。 在未确诊之前，患者往往经历漫长而复杂等求医之路，这个过程中需要家庭成员的照顾。 确诊之后的用药、手术、康复等过程，也需要家庭成员，特别是主要照顾者付出巨大的心力。

“这七年整，是我一个人照顾的，我也担心媳妇儿子都有意见，但是她是我闺女。 现在我有病了，我得糖尿病几年，我没法照顾她。 她闺女长大了之后，现在就是她闺女其实有承担一部分照顾她的，就帮她洗澡什么的。”

——P-HN-30

“当时要从单位请假来照顾（我老婆，笔者注），前前后后，就她生病到现在，前后请了差不多半年。 就直接跟领导说，我老婆有病，然后请假回去照顾。 她严重的时候就是要，就是行动什么的都需要照顾，上洗手间，洗澡什么的，她最厉害的时候脚没劲吗，行动不方便。”

——P-HN-18 丈夫

经济支持

重症肌无力对家庭影响最直接的方面，就是家庭在医疗方面经济支出的增加，特别是对本身没有太多收入的未成年病友或没有稳定的经济来源的成年病友。 此外，由于重症肌无力患者中女性居多，在患病前，部分女

性患者主要是家庭主妇，承担着照顾家庭的角色，没有稳定的收入。 患病后，则需要配偶甚至娘家从经济方面的支持。 对于成年男性患者而言，可能此前是家庭经济方面的主要收入来源，患病后也需要家庭其他成员贴补。家庭如何调整经济方面对患者的支持，以及如何因此达成彼此的认知，使得患者不会因为经济支持产生进一步的心理负担。

“我父亲有病是 2000 年，我去借钱，谁都不害怕，我借了十万八万的，有人给我拿，当我有病的时候，我跟人家借钱，人家不会借给你，因为你没有偿还能力，你靠两个老人怎么还，很正常的那个时候。现在我主要靠父亲的退休金，一个月三千多贴补。”

——P-LN-06

“因为我姐的身体也需要，也需要需要金钱，需要金钱跟跟一些人去照顾。 我两个姐姐就留在家里照顾，然后我哥。 我哥就要出去赚钱。 然后给我姐治疗。”

——P-BJ-09 家人

情感支持

如前所述，病情会对病友对心理产生很大影响，由于疾病的慢性与波动性的特点导致这种影响往往是持久的。 由患病带来的焦虑、愧疚、抑郁等情绪会贯穿整个病患旅程，家庭成员也会因为患者的心情而受到影响。 但作为患者的家庭，如何为患者提供适当的情感支持至关重要，因为这不仅有利于患者勇敢面对病情，也有利于支持患者面对未来生活的挑战。

“她那一开始跟我姐家，我不是接到我家了吗，接到我家后来我就啥都不寻思了，因为她那个没有啥（亲人，笔者注）了。 来到我家了，我就没有

啥想法，俺家我儿子小，那时候一准跟她干仗，我说儿子我说你别跟你二姐干仗，你看你二姐多可怜，妈妈爸爸都不在了，妈妈这几年还行，给你二姐接到咱家，我说你二姐过年过节她心里也难受。俺家你姨夫那人好，现在也说俺家 4 口人，有个姑娘，就说我外甥女。”

P-LN-01 阿姨

7）病友组织与地方病友社群

病友组织

确诊重症肌无力之后，多数病友会先后通过医生介绍、病友推荐、网上搜索、亲朋推荐等方式加入病友群，通过线上链接 QQ、微信群等方式，接触爱力重症肌无力罕见病关爱中心，与其他的病友建立联系，从而从不同程度获得相关的服务。从“爱力”机构的网站上可以看到，他们开展了咨询陪伴、康复教育、医患交流、救助帮扶、融合就业、社群赋能、领域推动等版块的服务和项目工作。

在我们访谈的病友中，很多病友都给予病友组织积极正向的评价，在疾病诊断后，越早能接触到病友组织的病友更有可能接受正确的诊断与治疗，从而少走弯路。同时，当治疗过程中遇到断药危机、医疗救助等需求时，病友组织能通过发声，动员药企、医院等利益相关方，解决病友亟需的医疗需求。此外，病友组织也关注病友在康复、心理、就业等方面的需要，并通过赋能培育等方式帮助有需要的病友。

“我觉得，要是说咱大白话，就心理老师，就是给我们讲讲那些心理的那些东西，让我们就是能走出来。能从自己那个就得病之后，不都会挺悲观的吗？从那里走出来。”

——P-BJ-09

地方病友支持

病友们通过病友组织线上的方式链接，之前患病带来的孤单感和外人无法体会的痛苦很快在线上找到共鸣。除了线上交流之外，病友组织也会将同一地区的病友聚集到同一个病友群，方便相互之间的联系沟通和彼此支持，通过支持当地的病友骨干来支持病友们形成线下社群，例如支持当地病友群线下交流聚会等。由于地域因素，病友们往往很快建立联系，彼此更加熟悉。因为处在同一地域下的医疗需要，往往能够在诊断、治疗、康复、心理与情绪上彼此支撑。病友的地方社群由此产生的支持网络也会给病友提供实实在在的支持，这种支持让他们看到彼此，从而能在日常生活中找到社会支持网络。

“因为当初手术是一个那个病友去抬的我，我记得特别清楚，他在我的右手边抬我，我还拽着他嘛，姓韩，叫韩＊＊，他是长春市内的，这得记一辈子(笑)。当初就在东北群里头唠嗑，还有那个病友的爸去抬的我，因为我这比较胖。大伙去抬的，抬出来。”

——P-LN-06

16. 其他问题

1）医患沟通

患者角度

本研究在与重症肌无力患者及医生的访谈中发现，医患沟通为阻碍患者治疗和康复的一大难题。本章节首先从患者的角度梳理医患沟通难的

原因。

首先，多位患者表示其医生在与自己的沟通中，并未能向自己解释清楚重症肌无力的致病机理，即为何自身免疫系统会导致肌肉无力的现象。同样，医生也未能解释患者为何要服用、增减或更换某种药物，或为何要采取胸腺切除手术、丙球冲击或血浆置换等治疗方式。这一方面是医院繁忙的看诊系统所致——患者普遍认为自己每次看诊与医生的见面时间过短，无法深入向医生询问和了解自己的疾病管理进程。另一方面，患者自己也承认自己不具备足够的医学常识，“不知道该向医生问什么问题”。这种沟通的困境阻碍了患者对自身疾病的理解，也部分导致有些患者未对疾病管理引起足够的重视，因而未能遵医嘱进行治疗。

“医生和患者之间好像是隔离的，他们怎么研究这个病好像和患者之间没有沟通一样。”

——*P-XJ-03*

其次，部分患者对医生没有足够的信任，这也导致医患沟通的困难。信任欠缺的背后有复杂的原因：因为大多数患者没有长期了解其病情的主治医生，而是因为外出务工、挂号困难等原因随机寻找医生看诊，所以医生对其病史和过往用药仅有有限的掌握，这导致有些医生所开药物并不适用于患者当前的病情，从而引发病症波动甚至复发，继而引发医患关系的不和。在这种情况下，患者或是尽量避免与医生的沟通，或是认为单个医生的意见不具备足够的权威，而根据自己的认识选择性采纳医生的药方。

“感觉在某市治疗那次也是一次很大的打击。后来就好像是放弃了，觉得不可能再找到好医生了。就在家里这样就可以了，然后我自己再在网上自己学中医，然后自己买点儿中药自己。在外面的话治也治不好，花钱

也多。”

——P-XJ-03

研究员：“那您为什么没有听＊＊医生的意见，换硫唑嘌呤或者他克？”

受访者：“因为那个咋的之后，我不相信医生权威，我一般要有重大药物调整的话，我会多问几个大夫，完事儿，把这几个大夫意见放一块儿。我就这样的习惯，就个人习惯造成的就是。”

——P-LN-06

最后，病人的依从性不够会加重医患关系的紧张，病患常描述总感觉医生在“责备”自己未能按时用药，这种责备的语气会进一步使病患畏惧与医生的沟通，减少复诊次数，引起恶性循环。

“比如那个＊＊医生就脾气不太好，他就是那种强制性的，就说我要你怎么怎么样，闹得不是很愉快，他老觉得我不听话。后来他也就没怎么管我了。”

——P-FJ-11

医生角度

在我们对神经内科、胸外科、中医的访谈过程中发现，医生也认为医患沟通是影响病人依从性及疾病管理过程中的重要因素。研究过程中，我们发现，从医生的角度而言，影响医患沟通的原因主要包括以下因素：

首先，中西医关于重症肌无力的解释是完全不同的。西医可能就疾病发病机制、致病因素、诊断治疗、用药解释等方面来解释，而中医则偏向于从阴阳角度来解释。或许这两方面的解释构建了病人对于疾病的理解。

其次，早期病人的来源主要是眼科、胸外科、其他科室转诊，这导致病人获得正确的诊断与治疗之前，已经经历的误诊漏诊等曲折的诊疗之路，对医生作为整体的信任感就有所影响。随着近年来直接挂号的病人增多，这

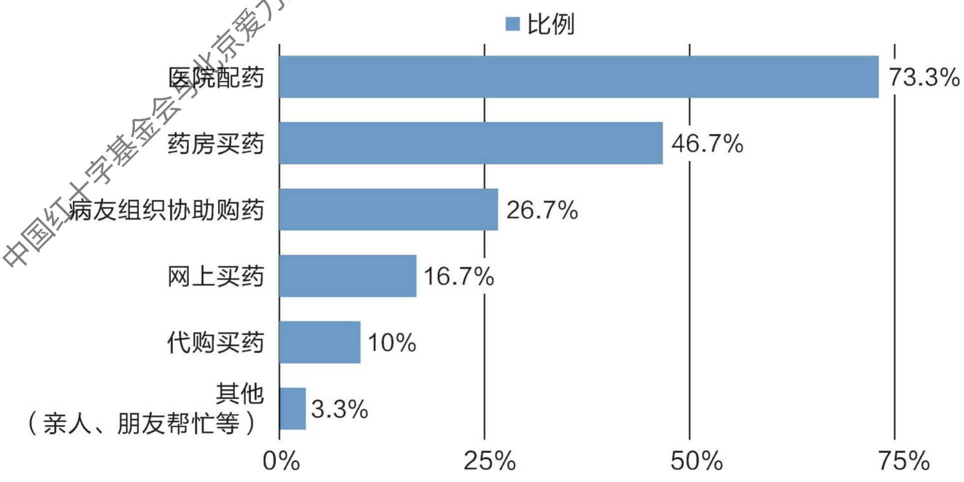
有利于医生与病人之间的信任关系的建立。

最后，医生与病人的沟通态度和技巧很重要。不少医生受访者提到，依从性比较好的患者，在于医患之间平等的沟通，能否以平实的语言让病人了解自己的身体状况，医生是否为病人详细解释了检测治疗方案等，都是影响病人依从性的关键因此。

我们发现，由于疾病的慢性特点，加上我国医疗体制下的挂号难等问题，不少病人通常在后期就认为自己可以“久病成医”，不再定期挂号复诊。然而医生们则认为，目前存在病人很难就病情定期向医生反馈对情况，挂号难、路程远、诊疗分割等难题，使得医生无法完全掌握病人的病情及用药反应。

2）购药渠道

重症肌无力患者购药渠道不一。大多数患者都有在医院或药房配药的经历，但有时因为药物缺货或价格太贵的原因，患者需要采取更多更主动的措施来购买到自己需要且价格合适的药物。在本次访谈中，患者所提到的购药方式如下。

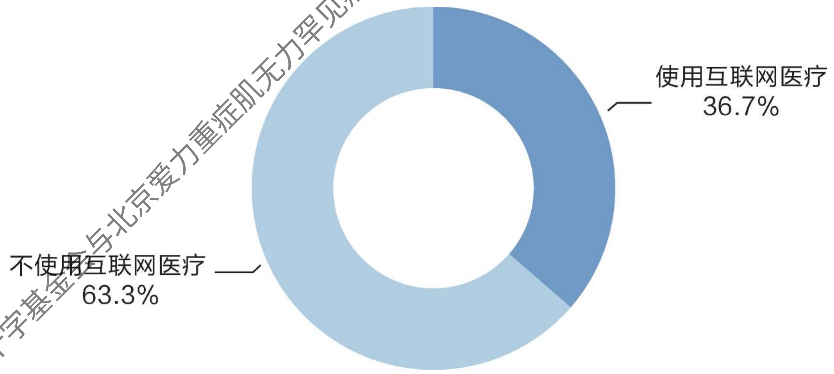


患者购药渠道分布（共 30 人）

值得注意的是，多位患者提到溴比斯的明曾经有几度在国内大片区域缺货，病患不得不通过患者组织集体向药厂或药品监督管理局反映此缺货问题，并积极寻找新的购药渠道。另外，他克莫司等三线药物对大多数患者来说都经济负担过重。多位患者提到他们会多方比较各个渠道的药物价格，并选择便宜且“看起来正规”的药物。患者并无渠道验证网络购买或通过药代购买的药物是否为正品，这也可能会为医药市场带来一些隐患。

3）互联网医疗

因为挂号、异地就医、寻找合适的主治医生等方面的困难，许多病患正在逐渐尝试并接纳互联网医疗的方式帮助自己定期复查。在本研究访谈的患者中，有 36.7% 的患者曾经采用或正在使用好大夫等平台进行互联网医疗，不过这些患者都认为互联网医疗可以作为辅助医院看诊的手段，而不能成为自己所依赖的唯一看诊方式。



患者互联网医疗使用分布（共 30 人）

使用互联网医疗的患者大多都认可其带来的好处，例如能够解决挂号难的问题、能够更方便地向名医问诊、免去异地就医舟车劳顿之苦、方便向医生主动提出问题等。但也有患者提到，远程问诊相比医院问诊挂号费更贵。同时，相比西医来说，中医更需要“望闻问切”，所以互联网医疗仍旧

无法替代实地诊疗。

“互联网医疗就是你可以问挺多次的。其实你去医院的时候，挂个专家号也得五六十或者一百块，然后有的时候可能医生也就几句话就把你敷衍掉了。相比而言，互联网我可以问得仔细一点，就是他能看一下你的片子，然后跟你说一下。”

——P-JS-13

“好大夫的话，我用过一次。当时是遇到什么情况了，我记不太清楚了，可能就是病情反复了。那个好大夫挺贵的，我挂上号之后他就直接打电话过来了。说实话，当时也没有解决什么问题。就是你电话说几句话，他不能给你下结论，也不能给你开药，他不能全面地了解你。”

——P-HN-27 妈妈

4）患病对生育的影响

重症肌无力的女性患者可以正常怀孕和生产，但是这个过程当中不可预测的因素对病情的疾病管理、产程的孕期管理来说都有重要影响。如前所述，我们访谈的20位女性患者中，7位是发病前生育的，6位是发病后生育的。在发病前生育的患者的表述中，其中有6位我们认为其发病的触发因素可能与怀孕生产和坐月子带来的身体情况改变有关，其中还有女性患者因此引发危象。

产科医生对重症肌无力对重症肌无力疾病管理的知识与神经内科、胸外科医生对产科相关知识的认知是有代沟的。尽管，在医生的访谈过程中我们发现，国内不少在重症肌无力方面有着深层积累的医院都注意到女性重症肌无力患者的生育需求以及由此引发的疾病管理挑战，也有医生在倡导相关的多学科管理，例如湖南某医院等。但是对于基层患者经常就医的

基层医院而言，还是存在认知不足的问题。

5）难治型

虽尚无研究结论，但本报告希望对重症肌无力“难治型”这个概念提出一些探讨。本研究认为“难治型”是一个本质上有争议的概念。对于医生来说，“难治型”重症肌无力病人有较为详细的医学操作定义：（1）对常规疗法没有充分反应；（2）在没有临床复发或需要持续的挽救性治疗（例如静脉注射免疫球蛋白或血浆置换）的情况下，无法减少免疫抑制治疗；（3）免疫抑制治疗的严重或无法忍受的不良反应；（4）限制使用常规疗法的合并症；（5）即使在治疗期间也经常出现肌无力危象。然而，对于患者来说，“难治型”更多是一个社会建构的概念。在访谈过程中，多位患者提及自己病情反复治不好的原因是“我是难治型”，然而这些患者大多仅为 AchR 抗体阳性，并非医学定义上的难治型。导致其病情反复的原因，更多在于其弱势社会经济条件带来的稳定治疗困境。因此，该报告提倡医学及公共卫生领域除关注临床操作定义上的“难治型”病人以外，也要关注这类社会建构的“难治型”病人，给予其适当的政策和社会保障支持，帮助他们更有效地治疗和康复。

第四部分
医生视角下的重症肌无力诊疗现状

17. 医生访谈

我们访谈的 7 位资深医生专家在行业内有着丰富的重症肌无力诊断、治疗与研究经验。 我们了解到，虽然我国的基层医生关于重症肌无力的诊断与治疗的认知与技术水平在不断提高，关于重症肌无力的症状、诊断方法、检测手段、用药基础等方面都已经有所掌握，能够让患者在治疗过程中少走弯路。 然而，在达到这个门槛之后，如果区分更进一步的用药、手术、症状细分等知识是面临的进一步挑战，例如如何分辨单纯的眼肌型与全身型患者，反而对经验丰富的医生而言，眼肌型的区分要难于全身型。

我们知道在临床医生的培养过程中，关于重症肌无力的论述在相关的教科书里仅占一小部分。 因此在临床训练过程中，对医生进行重症肌无力的训练与培养就十分重要，如何通过在职训练与多学科合作来规范与提升相关对诊疗水平是我们面临对又一挑战。

此外，医生与患者认为中医的作用是不同的。 多数医生认为西医是诊断、治疗、抢救的手段，中医在于控制西药的量并且平衡其副作用带来的影响。 相反，患者会把中医的角色扩大化，甚至“病急乱投医”。 这是我国背景下非常特殊且有意思的现象，值得进一步研究与探讨。

第五部分
总　　结

18. 调研总结

本研究采用序列混合方法（sequential mixed methods），通过横断面调查为主的定量研究，收集了有效问卷 1020 份。通过以深度访谈为主的定性研究，对 26 位重症肌无力患者，7 位重症肌无力患者家属的访谈和 7 例医生的访谈，描述了我国重症肌无力患者的生活质量，总结出中国重症肌无力患者从发病到确诊再到治疗与康复这个过程中的典型病患旅程，以期达到公众和政府能了解重症肌无力疾病，了解重症肌无力对患者、家庭和社会造成影响。同时，我们呼吁更多临床医生能够识别和诊断重症肌无力，希望能有更加有效的治疗药物上市；帮助患者解决治病难、用药难和用药贵的问题。具体而言，我们总结出如下几点，供有关方及公众思考：

首先，许多病人仍面临确诊的困难，而确诊的关键节点在于是否可以准确定位到神经内科。为了促进更多重症肌无力患者快速确诊并开始治疗，三方面的知识普及应当得到加强。首先是对眼耳鼻喉科及其他相关科室医生的知识普及，以帮助更多首先到这些科室就诊的重症肌无力病人能得到准确及时的转诊；其次，对中医的知识普及也应当得到加强，从而中西医结合治疗能更有效地展开；最后，相关公共卫生健康部门也应加强对公众的知识普及，提高公众对现代医学知识的认知，从而使各类患者都能更容易更及

时地找到就诊科室。

其次，防止复发的关键点在于患者的用药、合并症及生活与情绪管理。 在用药方面，患者的用药依从性仍有待较大提升。 为改善这方面的问题，一方面，患者的对疾病管理的认知有待提高，患者组织也应加强对患者的医学常识教育；医患之间的信任关系尤为重要，医生应该尽可能在听取病人反馈的基础上长期帮助病人调试最合适的治疗方案。 对于一些难治性病人或居住地偏远的病人，应探索由专人进行长期随访的可能性。 而对于处在特殊时期（例如孕期和产褥期）的病人，医院也应当加强产科与神经内科的合作，以帮助产科对这类患者要给予特别支持方案，并积极推进多学科医生间的合作。 除此之外，患者日常生活与工作的安排，以及心理和情绪的调适，也应当得到足够的重视。

第三，许多重症肌无力患者并非医学定义上的“难治型”患者，他们的病情之所以“难治”，更多是由于社会结构上的弱势地位导致。 为帮助这类社会建构的“难治型”病人获取更持续更稳定的治疗，相关政策制定部门应当在社保医保上对其加以支持，推动更多二三线药物纳入医保范围，在全国各地完善及推广“重大疾病特殊门诊”的报销政策。 各地医院也应当持续推进互联网医疗方案，帮助偏远地区及经济欠发达地区的病人更方便地接触优质医疗资源。 此外，患者组织也应当在就业培训与辅助、心理咨询与调适、病友社群建设等方面给予重症肌无力患者更多的关注和支持，从而减轻患者的经济负担，为患者提供全方位的关怀。

第四，提高医生对重症肌无力的关注，促进诊疗过程的完善，推动诊疗指标标准化。 近年来，随着医生群体对罕见病诊疗过程的提高，重症肌无力患者如果能准确找到相关医院的神经内科，进行确诊的难度有所下降。 然而，我们也看到，由于重症肌无力发病的初始症状不一，部分患者会去到眼科、耳鼻喉科、五官科、妇产科等其他科室，其他科室的医生能否对这些症状进行识别以及转诊，是患者少走弯路，及时治疗病情的关键，因此我们呼吁其他科室的医生也应该对重症肌无力疾病有基础的认知。 此外，就神

经内科而言，我们发现不同类型的医院治疗手段略有不同，我们呼吁神经内科医生根据诊疗指南，丰富和改善治疗方案，与患者进行治疗过程中，详细解释病情，加强患者自身对疾病的了解，推动病人早就诊，早确诊，并得到适合的治疗，从而进一步减轻患者的疾病负担。

第五，创新治疗方案，引进相关新药。 患者目前可负担的治疗药物相对单一，主要集中为抗胆碱酯酶抑制剂，激素，免疫抑制剂等，由于药物每日服用的不方便性及考虑到药物对身体带来的各种副作用，患者的药物依从性较差（MMAS-8 仅为 4.7），这也是导致病人复发的主要原因之一。 病人和家属都迫切地希望有创新的治疗方案，评估新药及新的治疗方案与传统治疗方案的对比而言的效用，采用对病友身体及社会功能恢复更好的方案，以帮助病友更好地控制疾病，更好地融入社会。

第六，通过社会保障制度减轻患者及家庭经济负担。 疾病对患者的生活质量带来巨大的影响，受到疾病影响，多数病人无法正常工作学习，约 41%的病人无经济收入，50%的家庭的月收入在 5000 以下，经济负担较重。 近年来，国家及地区层面针对罕见病的诊断与治疗出台了多项适切的支持政策，重症肌无力患者需要终身服药，药品的费用、药品获得的可便利性会很大程度影响患者的依从性。 因此，调整患者在不同地区药物使用及报销政策，通过药物及报销政策提升药物报销的可获得性，通过社会保障制度减轻患者及家庭的负担至关重要。

第七，通过社会支持帮助患者及家庭提高生活质量。 疾病对患者的生活质量带来巨大的影响，生活，工作及学习的计划皆要以疾病为考虑的中心，部分患者丧失独立做事和能力，还有少量患者出现说话困难及行走困难。 只有 41.7%的患者完全有自理的能力。 对患者自身及家属都带来负担及困扰。 80% 以上的病人会累及四肢肌和躯干肌，对生活带来很大的影响；部分病人会累及咽喉肌和呼吸肌，会增加危象的发生。 MG 患者应该积极干预，坚持治疗，防止疾病的复发及进展。 此外，患者的精神健康状况也不容忽视，由于疾病给个人和家庭带来的负担，患者普遍心理负担很

重，抑郁焦虑的情况普遍发生。因此，我们倡议政府应该出台更多的社会支持措施，包括心理健康、社区照顾、康复等，帮助患者提升生活质量。

第八，关注女性病友的生育健康。重症肌无力患者中，女性患者比重较高（>70%），且发病年龄较早。在访谈中，不少女性患者提及生病对生育的影响，由于生育管理及控制不足，导致疾病复发的情况时有发生。因此，医院相关科室是否能开展多学科合作，提供更为安全有效的生育支持方案支持她们的生育需求，关注新生儿重症肌无力现象，更重要是如何控制好病情的情况下度过怀孕生产的阶段。

第九，培育民间力量，支持患者组织发展。罕见病群体人数少，面临着诊断与治疗的可及性难题与社会支持匮乏等困境，患者组织与患者社群有着天然的连结，在支持重症肌无力等罕见病社群的诊断与治疗的过程中发挥着独特的作用。患者组织不仅能帮助重症肌无力患者快速准确地找到对应的医院科室与医生进行适切的诊疗，也能在协调医患关系、领域推动等方面团结患者社群进行相应的价值倡导，推动以患者为中心的医疗体系的构建。此外，患者组织对重症肌无力社群进行的咨询陪伴、康复教育、救助帮扶、融合就业、社群赋能等板块的服务，有效地帮助患者个人及家庭应对因疾病导致的社会困境，从而帮助社群更好地与疾病相处，转介相应的资源来帮助患者应对疾病对个人与家庭带来的挑战，更好地融入社会。因此，政府及民间力量应积极培育患者社群，支持患者组织发展，引入相关资源及培训来提升患者组织服务社群的专业性，从而进一步提升罕见病社群的整体生活质量，更有效地推动一个健康公平的社会。

附　　录

量表1：中文版8条目Morisky服药依从性量表①

	是	否
1.您是否有时忘记服药?	○	○
2.在过去两周内，是否有一天或几天您忘记服药?	○	○
3.治疗期间，当您觉得症状加重或出现其他症状时，您是否会不告知医生而自行减少药量或停止服药?	○	○
4.当您外出或长时间离家时，您是否有时会忘记带药?	○	○
5.昨天您服药了吗?	○	○
6.当您觉得疾病已经受到控制，您是否停止过服药?	○	○

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① 引自：秦佑鹏，杨一君，姜葶蔚．药物依从性问卷介绍和思考[J].《健康世界》2019年第10期．

	是	否
7.您是否觉得坚持计划有困难?	○	○
8.您觉得要按时按量服药很难吗?	○从不 ○偶尔 ○有时 ○经常 ○所有时间	

量表 2：合理用药自我效能量表（self-efficacy for Appropriate Medication Use Scale，SEAMS）①

发生以下情况时，您按时服用或者使用药物的信心是多少？（SEAMS）

	没有信心	有点信心	非常有信心
1.当医生更换你的药物时	○	○	○
2.当你发现重新买的药物与以前的药物在服药方法上有差异时	○	○	○
3.当你每天需要服用几种不同种类的药物时	○	○	○
4.当每天服药次数大于 1 次时	○	○	○

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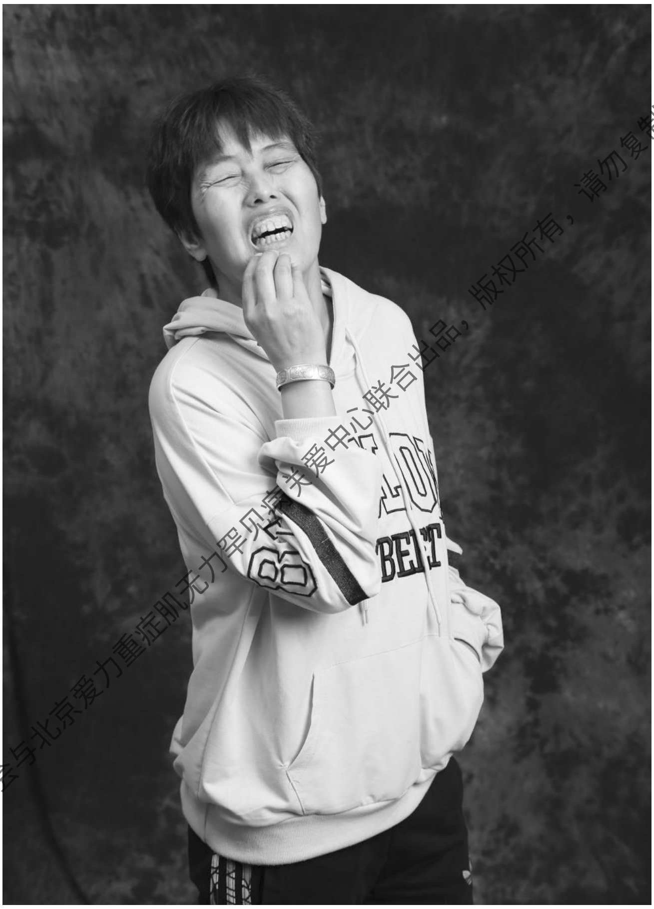
① 引自：董小方，刘延锦，王爱霞.合理用药自我效能量表的汉化及信效度检验[J].护理学杂志：综合版，2015(6)：47-49.

	没有信心	有点信心	非常有信心
5.当你出门在外时	○	○	○
6.当你某天很忙时	○	○	○
7.当药物产生不良反应时	○	○	○
8.当没有人提醒你时	○	○	○
9.当服药程序比较麻烦时	○	○	○
10.当你日常活动计划被打乱时	○	○	○
11.当你不太确信服药方法时	○	○	○
12.当你不太确信在哪个时辰服药时	○	○	○
13.当你患其他疾病时(如受凉或感冒)	○	○	○

患者故事



鞠秀芹，黑龙江人，病龄31年，患病后父母与弟弟相继离世，曾一度认为自己愧对家人，是社会的负担。因为小姨的关爱及爱力与社会好心人的帮助，逐渐找回信心，立志成为一个对社会有用的人，如今成为一名帮助病友的全职公益人。

患者感言

朱霞，来自山东聊城，49 岁，病龄 6 年：

病后的痛苦和绝望随着时间可以慢慢消退，而被社会忽视和冷落的痛时时折磨着我，令我窒息。 陌生的你愿意给我一个微笑和拥抱吗?

丛树文，来自辽宁铁岭，54 岁，病龄 12 年：

自有病到现在，经历了十二个春秋。 在这十二年当中，有悲伤，有失望，有泪滴，也有欢笑。 从生活不能完全自理到现在，经历了太多太多。有时候自己摸自己的头，知道自己还活着，眼泪不禁夺眶而出。 活着真的太难了。 从长途跋涉到四处求医，想想曾经被误诊和曾走过的弯路，再想想与死神擦肩而过，这一切历历在目，真是叫人胆战心惊。 忘记过去，展忘未来，希望就在眼前。 我真心希望能研究出更好的新药、让更多的病友看到光明与希望吧!

汪恩宇，来自江苏省宿迁市，30 岁，病龄 1 年：

一年半，我从眼肌型到全身型再到延髓型。 几个月前还以为能跟个正常人一样，现在每天早晨很明显的感觉到漏气，吃一口饭咽下去然后像小鱼吐泡泡一样漏一点气出来，又不像打嗝那样，很不好受! 我挺苦恼的，租房费、医药费，工作又经常请病假没工资，捉襟见肘，生活质量越来越差，不敢想以后，现在可能是我最好的状态了吧。 希望能报销中药费，尽量减少生病的痛苦。

赵宝平，来自北京，45 岁，病龄 33 年：

有时候，困境是生活给予我的一种馈赠。 我被禁锢在这方寸之地，没有生活的奔波劳累之苦，也没有能胡吃海塞的身体和能力。 我觉得国家可以立法，尊重那些实在无法治疗，深陷病痛中的人实施安乐死。 我这无儿无女的人，等到生命的尽头，希望自己可以选择体面的离开。 能活的时候，我会好好的享受当下。

任元春，来自上海，45 岁，病龄 8 年：

我在 2014 年底患上重症肌无力，此后才知道有关人体免疫系统的一些知识，得知患病前的长期亚健康其实就是身体的警告，如果及早通过改善饮食、改良生活方式、关注心理健康，是有可能预防疾病的发生。 即便患病后，这几个方面也应该是病友康复长期着力的方向。 希望舆论引导市民关注健康生活方式、关注心理健康。

王淑芳，来自黑龙江绥化，66 岁，病龄 15 年：

我多么希望能不再用激素和免疫抑制剂，年龄大的人身体已经退化，还要常年服用激素和免疫抑制剂，我现在是高血糖、高血脂、高血压、骨头退行性病变、肾损伤。 但这些维持的药不吃还不行，真的希望能有对症的特效药，救救孩子们和年轻的患者。

白萌，来自湖北襄阳，35 岁，病龄 6 年：

生活的磨难并不仅仅只来自于疾病，患病时年纪尚轻，主要的困扰是那段没有确诊的日子里，整天没有力气，做事情心有余而力不足，总是躲躲藏藏怕被人发现被笑话，而这样的日子随着疾病的确诊和接受治疗，也慢慢远去。 再后来才发现，那段艰辛的日子，并没有代表生活所有的苦难，也并没有所谓的苦尽甘来，而这一生注定只能是负重前行。

李双新，来自湖北襄阳，32 岁，病龄 3 年：

疫情 3 年，我也得病 3 年了，从刚开始的无助，无奈，到现在的坦然接受勇敢面对，也是经历了许多难熬的夜晚，家人的鼓励，朋友的支持，病友的安慰，医护的细心照料换来的。 虽然得病是不幸的，但这一路遇到了许多帮助我的人，是幸运的。 疾病带来身体上的痛苦，我们无法改变，内心世界的快乐我们还是要始终保持。 希望我们的病友都越来越好，期待我们这个罕见病群体能被更多的人关注，也期待更多的人为爱勇敢发声！

彭燮，来自北京，43 岁，病龄 3.5 年：

每一个罕见病友可能都反复问过一个问题“为什么是我生这个病?”我也一样。 可后来我意识到“为什么不能是我?”芸芸众生中，除了少数大奸大恶之徒，其他纯良百姓，又有谁是应该要得这个病呢?！ 生病，是我们人生中无数意外和困难之一，从天而降，猝不及防。 没有生这个病的人，他们也会面临各种痛苦和无奈。 人生就是如此，再苦也得忍着，因为这就是属于你的一辈子。 我们能选择的就是，过好这一生，让自己和家人多点好的念想。

胡茜，来自广东韶关，28 岁，病龄 14 年：

生活，我想义无反顾地奔向你，想要张开双臂拥抱你，想要抬眸凝望你，想要轻声呼唤你，想对你微笑……想要做的这些别人眼中轻而易举的事情却无法做到。 备受煎熬，身心皆无力。 可因为爱，每一次呼吸都是充满勇气且拼尽全力。 我相信，终有一日，我们会和许许多多的人一样，健康有力量，轻松表达自己内心的欢喜，不再受到其他人异样眼光。

翟秀珍，来自内蒙古呼和浩特，68 岁，病龄 17 年：

我是一个要强的人，重症肌无力让我从过去的“全能冠军”变成了“无能为力”，心情不好到了冰点，极度郁闷。 后来，找到了爱力，和病友在一起我看到了希望，感受到了活着真好。

我现在的病情越来越轻，身体越来越好，我不打扰家人，生活完全自理，还能照顾老伴，形成了良性循环。感谢家人，感谢爱力，感谢重症肌无力，给了我再次站起来的力量，使我内心更有能量！

周琪，来自黑龙江黑河，53岁，病龄6年：

希望国家完善重症肌无力疾病的医疗保障体系，将我们的用药列入医保名单；希望偏远地区重症肌无力患者群体能得到及时诊治，减轻外地就医的经济压力；希望有一个重症肌无力群体的就业平台，提供合适的工作岗位为患者群体减轻家庭经济压力，也为自己的人生画上重彩的一笔，为社会做出贡献。

王鹏，来自辽宁，44岁，病龄16年：

患病16年，曾经几次危象几次住院，家里经济已经艰难，而我和病友每天必须吃的药“溴比斯的明”却一直在涨价，还不是很好买，还有免疫球蛋白，虽然已经纳入医保报销目录，但去医院却怎么不给报销呢？ 有一年我住院在ICU里10多天，对一个常年有病人的家庭来说，真是雪上加霜，住院的钱都是妈妈带着我三岁的女儿去一点一点的借来的，虽然我是成年人，可哪一个母亲也不希望孩子被疾病带走，孩子是她的天，天不能塌下来。 希望我将来能照顾好母亲和孩子。

黄霞，来自江苏泰州，44岁，病龄10年：

总觉得老天喜欢捉弄我，喜欢和我开玩笑，所以认为没有过不去的艰辛。 虽然为了活下去需要付出多少努力承担多少压力，选择坚持也要经历无数次思想斗争，却不敢让任何人知道，怕无情，怕失意，怕鄙视，怕冷落，怕落寞……不敢淋漓尽致表达自己的哀与乐，懦弱再痛也只能掩埋心底。 我不敢公布病情怕没有了生活来源，怕单位不再要我。 希望哪天可以光明正大地吃药，再也不用伪装，偷偷摸摸吃药维持，生活可以轻松一点！

周艳霞，来自湖北武汉，51 岁，病龄 23 年：

我是 1999 年确诊为重症肌无力的，这 23 年，经历过无数次的治疗一复发一治疗，从刚开始的消沉、焦虑、茫然，到现在的坦然面对，我走了很长的一段路。 我正在努力成为一名可以帮助病友的关怀员，用陪伴，自助助人，与疾病和平相处。

刘红兵，来自河南林州，48 岁，病龄 13 年：

我是一位确诊患有 13 年病龄的重症肌无力病友，从 2007 年开始出现重症肌无力症状，到 2009 年确诊，一路走到现在，从当初儿子上小学一年级，到今年儿子大学毕业，整整 16 年！ 是家人们的长期坚守和陪伴，和自己的不放弃，是爱力和爱心人士的捐赠和帮助，是热心病友们的生命影响生命，给了我力量！ 苔花虽然小，也学牡丹开。 期待更多社会各界人士关注重症肌无力，给患者提供帮助，包容理解，让更多病友重返社会！

李迎进，来自天津，67 岁，病龄 8 年：

期待爱力能帮到更多病友，让我们共享炽热情感的关怀，在这个大家庭里友爱互助，更有利于患者的疗伤，因为我们不但需要医疗，也需要精神上的疗愈。 我也常听爱力开展的健康知识讲座和理疗方法，现在的病情相对稳定一些，也得益于治疗和这些方方面面的功效。 这是我的期待、需求，也是感悟和道白。

苏小珠，来自广东茂名，66 岁，病龄 20 年：

苏小珠是我的母亲，我是她儿子，我妈是个不识字不懂普通话的农村妇女，病了很多年，又因为这次新冠肺炎住院上了呼吸机插管，本来已经好转，但因为插管时间过长，管被痰塞住抢救了十几分钟才抢救回来，转进 ICU 后是昏迷状态，期待我妈能早日醒过来，平安出院。 新冠肺炎对重症肌无力患者来说很不友好，不但是易感人群，大多抗生素不能用，呼吸肌无力，更容易出

现呼吸困难，期待国家能在新冠治疗上对重症肌无力患者出台一些政策。

陈文花，来自广西崇左，43 岁，病龄 6 年：

我是重症肌无力患者，同时也患有干燥综合征、抗心磷脂综合征，中药加西药治疗，一个月中药费一千八左右，加上西药和复查，每个月花两千多。 我没有父母，没有成家，也没有好的工作和收入，日常帮亲属做些工作撑着，通常是慢病卡额度用完了只能把中药停了。 我的一年日常用药约 3 万，而慢病门诊卡只有 3500 的额度，我希望国家能把重症肌无力的报销额度提高一些，能保障我们长期地吃上药。

张涵溪，来自广西桂林，35 岁，病龄 7 年：

重症肌无力患者表面看着像正常人，没有肢体的残缺，但十分容易疲劳，体力差，症状时常反复，无法长期坚守岗位，成年人难以在企业全职就业，再加上药物的经济负担，生活质量很差。 如果有残疾症，或许可以进入一些福利企业，企业根据残疾情况酌情安排轻便可持续性工作岗位。 也可免费享受地铁，公交等交通工具，上车有人能给让座，还可享医院，车站优先排队购票的福利。

虽然重症肌无力患者在国家政策上是可以申请残疾证，但患者在调理稳定时，或休息后病情平稳了去办理，大多都无法通过审核。 重症肌无力病情是波动的，发病时生活不能自理，甚至穿不了衣服走不了路，尤其延髓型患者看上去很好，吃饭都会卡噎，呼吸困难，是无法工作的，没有劳动能力，却拿不到残疾证，办不了病退，建议国家考虑重症肌无力的病情特点，在评定时的政策按病种分型审核，给延髓型、全身型的肌无力患者评定为残疾证二级以上。

谢舒萍，来自广东深圳，34 岁，病龄 14 年：

重症肌无力这个疾病最好的情况是可以临床治愈，但不能彻底治愈，我

从 2009 年患病就长期服药，就拿基本每位患者都会长期服用到的溴吡斯的明片为例，这款罕见药物目前只有上海一家药厂进行生产。 当时溴吡斯的明单瓶售价 26 元，2023 年已经卖到 104 元，一瓶 60 粒，我最多时一天 8 粒，一瓶只能用 7-8 天，一个月需要 4 瓶，一年仅这一种药就高达 4 千多，还不包括其它免疫制剂的药费，重症肌无力在全国范围内仅有少数城市纳入了慢病或特病门诊，额度还很低，大多病友都很难承受，希望有关部门可以保障患者的日常用药！

薛亚柱，来自四川乐山，52 岁，病龄 5 年：

每天都在期待医学界发明一种根治重症肌无力的特效药，现在状态不是很稳定，心理负担沉重，患上这种病最大的感悟就是没有血缘关系的爱力工作人员、志愿者胜过了自家的亲人，虽未谋面，但爱力传递的正能量激励着我前行去战胜病魔！

丝路花雨，来自新疆昌吉回族自治州，64 岁，病龄 18 年：

期待在不远的将来不用吃药就能和正常人一样的生活，能出去逛街，去参加任何活动，时时刻刻都可以与人交流……有人会问这难吗？ 对我来说就是这么难，这药一吃就是十年八年，停了就复发，复发后比之前更严重，真的是病来如山倒，病去如抽丝，其他病以天以月为单位的好转，而我们则以一年二年为单位的好转一点，就为这一点好转也很开心，感到很不易了，希望再等一年……哪怕几年，不用吃药该多好！

付秀国，来自河南郑州，52 岁，病龄 10 年：

我来自河南郑州港区，从得了病变得很少出门了，更不喜欢说话，不喜欢和朋友相聚了，只想活在自我的世界里。 疾病不能过于劳累容易复发，在我们这里中药不能报销，西药溴吡斯的明片也从二三十涨到现在一百多，经济和精神的双重压力。 也希望大家不要因为眼皮下垂或表情僵硬就用奇

异的眼光看待患者，帮助病友回归正常生活。

姜显萍，来自黑龙江鹤岗，45 岁，病龄 10 年：

我是在偏远地区，身边没有人了解重症肌无力，也没人能理解我的一切症状。 除了病痛带来的身体痛苦，家人亲戚朋友的不理解更让我难过，那一段时间里真的有点想走极端，还好经过治疗现在平稳了，很知足。 虽然病后经济压力很大，但是有爱力和家人的支持，我已不再孤独，身边的人大多数也懂得了我的病情，不再用异样的眼光看我。 我也真正懂得了珍惜所有，爱惜生命，爱惜身边的人和事，珍惜拥有的每一天。

尚秋丽，来自河北衡水，48 岁，病龄 9.5 年：

重症肌无力，一群被折断翅膀的天使，希望国家将重症肌无力患者服用的常用药物纳入医保，提供更多的医疗福利，让他们以暴风雨般顽强的毅力，遨游天空，绽放苍穹！

马亚丹，来自河南洛阳，44 岁，病龄 7 年：

有一个群体叫“北京爱力重肌无力罕见病关爱中心”，有着同一种疾病，在不同的地方，病友们一直互相陪伴，让我总有一种信念，相信自己可以恢复，回到正常生活中。 我用了将近 4 年才走出来，也害怕过、无助过、痛苦过，所以也真真切切感受到现在很幸运，家人不离不弃的爱是我最坚实的后盾，自己的信念让我不断迈向前方。 我参加了病友关怀员培训，在陪伴病友中，感受病友之间的天然连接，信任，支持。 也学习到正确对待自己的疾病和破除心理上的阴影。

桑逢涛，来自山东枣庄，61 岁，病龄 11 年：

自从得了重症肌无力这个罕见病，仿佛人生跌入黑洞中，天天吃不完的药，月月往医院跑。 目前已经在黑暗中爬行了 11 年，期待爬行中看到前方

有光明，不再吃药，不再月月往医院跑，像正常人一样参加社会活动，还能为社会做贡献。

周爱玲，来自山东烟台，59 岁，病龄 5 年多：

我是全身型的患者，2018 年的时候气管切开，之后很长的一段时间由于脱离不了呼吸机，ICU 费用太高了，家人自费购买了家用呼吸机，由家人照料，一点点慢慢恢复，再慢慢到脱机，这几年一直在家人的陪伴下慢慢恢复，虽然无劳动能力但到现在能自己穿衣吃饭，家人们都很开心。希望更多好心人关注爱力，关注重症肌无力病人的困境！

付成伟，来自辽宁抚顺，42 岁，病龄 19 年：

人生就是一场修行，疾病就像是这修行中磨砺，通过努力征服疾病，这过程积累的经验将是永远的财富。 坎坷路走多了，以后能难住自己的事就会越来越少，生活越来越顺。 不去埋怨委屈，抬起头勇敢面对，总会有相同的伙伴一起前行。

金银贞，来自广东汕头，39 岁，病龄 22 年：

人的一生有太多太多的执念，与过去和解，与自己和解，与疾病和解，才能真正开始崭新的生活！

患者故事

刘红兵，48岁，河南林州人，少年患病，曾一度恢复，2008年复发后一直努力和家人一起对抗疾病所带来的痛苦。妻子每天给他订1份牛奶煮2个鸡蛋，自己和孩子却舍不得喝一口。重症肌无力不仅折磨着他一个人，而是一个家庭。

什么是重症肌无力

重症肌无力（英文名 Mvasthenia Gravis，简称 MG）是由神经－肌肉接头处传导障碍所引起的自身免疫性疾病，有晨轻暮重、经休息可缓解的特点。它没有医学遗传性，不传染，任何人可能在任何年龄后天发病。

重症肌无力能引起全身任意骨骼肌的无力和易疲劳性，所以会引发复视、眼睑下垂、无力举臂、无法抬腿、面肌僵硬无力、颈部无力、吞咽困难、构音不清、呼吸困难等各种大小肌肉群的无力感，影响患者全天任意场景下生活，像全身背着沙袋。

重症肌无力是“神经－免疫”类疾病，普通人往往将它有相类表现症状的疾病混淆，如“渐冻症”。 其实，严重的肌肉无力症状可以由很多疾病引起，严重的肌肉无力症状不一定就是“重症肌无力”这种疾病。 重症肌无力病情可逆，可治疗，相比类似症状的进行性疾病，它更像一片雪花。

据中国重症肌无力诊断和治疗指南（2020 版）的发病率推断，每年我国约有 9500 余患者新发病。 通常认为，重症肌无力的男女性别比约为 1：1.8。18－45 岁中青年女性、14 岁以下儿童、70－74 岁老人构成了三个发病高峰。

重症肌无力的中青年患者面临的社会问题最为严峻，除健康问题，还有更高的经济负担、照护、就业、婚姻情感、社会融合、家庭责任等一系列生存和伦理问题。

作为慢性疾病，重症肌无力波动性强，极易反复 ，病程终身相随，且有高致残风险和死亡威胁，需要长期进行疾病管理，同时，药物副作用引发各种合并症，许多“慎用药”会引起病情波动或加重，导致常见病也难以处理。

因此，见效快、副作用小、用药便利的新药将有效改善患者群体改善生存状况，同时用药的可及性、可负担程度是影响规范治疗的重要因素。

目前，我国仍有许多地区尚未将重症肌无力纳入门诊医保，药物不能被报销，或门诊报销比例过低，许多家庭、尤其成年患者失去经济来源后，无法坚持长期治疗，往往家庭高额负债，无法规范治疗甚至放弃治疗，也有患者不堪承受经济压力与疾病的双重折磨走上绝望之路。

北京爱力重症肌无力罕见病关爱中心简介

北京爱力重症肌无力罕见病关爱中心（简称“爱力关爱中心”）是 2013 年由重症肌无力患者创办、深度服务重症肌无力患者的非营利性社会组织，2021 年获北京民政局 5A 评级，成立以来以累计服务帮助患者与家属 13000 余名。

以“命运共同，感同身受”的初心，致力帮助患者提升治疗依从性和自我康复管理能力，降低危象和致残发生风险，实现与疾病和平相处，回归正常的社会生活。 开展了咨询陪伴、康复教育、救助帮扶、社会融合、社群赋能、领域推动六个模块工作，开展的项目如 ：615 重症肌无力关爱日、中国重症肌无力大会、爱力康复营、爱力康复包、爱力暖心、云鹿学院、社群关怀员体系、帮无力女性有力生活等，至 2022 年底，累计开展患者康复与赋能活动 400＋场，行业大型会议 6 次，大型患者调研 4 次，医护人员协作 300＋名，媒体发声 400＋次，自媒体发声 2000＋篇，资金物资捐赠患者 9000＋人次，帮助患者找到或买到药物 6500＋人次，发放康复手册 70000＋册，平均年咨询回复 10 万＋次。

愿景：每位病友与疾病和平相处

使命：推动社会对重症肌无力的认知，促进患者群体与社会的进步，改善患者就医和生存条件，促进医患交流与医学研究，帮助患者回归正常的社会生活。

工作目标：疾病治疗普通化、病友康复常态化、生活工作正常化、普及防治大众化。

口号：爱的力量，改变你我！

中国红十字基金会简介

中国红十字基金会成立于1994年，是中国红十字会总会发起并主管、经民政部登记注册的具有独立法人地位的全国性公募基金会。中国红十字基金会以“守护生命与健康，红十字救在身边”为使命，致力于成为“中国最具行动力、最具创新力、最具影响力的基金会”，宗旨是弘扬人道、博爱、奉献的红十字精神，致力于改善人的生存与发展境况，保护人的生命与健康，促进世界和平与社会进步。

2008年、2013年、2018年连续三次在全国性社会组织评估中获评"5A级基金会"。2010年、2015年、2021年连续三次荣获民政部授予的“全国先进社会组织”称号。截至2022年，中国红十字基金会累计公益支出超过100亿元人民币，人道救助和人道服务覆盖群体超过1000万人次，先后荣获7次中华慈善奖，共有8个公益项目获奖，包括：红十字天使计划、中国红行动、嫣然天使基金、春雨行动、小天使基金、“心拯救，一包药”、“新站·新健康”博爱卫生站、字节跳动医务工作者人道救助基金等项目。中国红十字基金会先后荣获中共中央、国务院、中央军委授予的“全国抗震救灾英雄集体”称号，获得中央和国家机关“四强党支部”“创建模范机关先进单位”“先进基层党组织”“五四红旗团支部”等称号。中国红十字基金会是首家通过ISO9001质量管理体系和SGS全球社会组织对标审核双重认证的基金会，获评“中国红十字会抗击新冠肺炎疫情先进集体”“年度透明基金会”“自律透明奖”“年度榜样基金会”“公募基金会公众影响力榜”第一名等多项荣誉。

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2022 Health report of patients with myasthenia gravis in China

2022中国重症肌无力患者健康报告

To Build A Life With Humanity And Dignity 创造人道和有尊严的生活

Aili Myasthenia Gravis Care Center
北京爱力重症肌无力罕见病关爱中心

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中国红十字基金会
CRCF CHINESE RED CROSS FOUNDATION

Health report of patients with myasthenia gravis in China

Initiated by: Chinese Red Cross Foundation

Initiated and implemented by: Beijng Aili Myasthenia Gravis Rare Disease Care Center

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Data contributed by：

961 patients

92 family members

7 doctors

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1. 2022 Health report of patients with myasthenia gravis in China；
2. Speech titled “Understanding Patient Needs through the ‘2022 Health report of patients with myasthenia gravis in China’.”
3. Speech titled “2022 Health report of patients with myasthenia gravis in China.”
4. Speech titled “Interpreting the Release of the ‘Health report of patients with myasthenia gravis in China’.”

Qing Zhao: Founder and director of Beijing Aili Myasthenia Gravis Care Center (Aili Care Center). She was diagnosed with myasthenia gravis in 2002 and began to help other patients in 2006. In 2013, she co-founded Aili Care Center with other patients. Following the idea of "helping others to help self and helping self to help others," she has inspired more and more patients to be involved in the work of public welfare.

Preface

During the writing of the report preface，Professor Xu passed away due to illness. We are extremely saddened about this. Professor Xu had devoted his whole life to the construction of neuroimmunology in China and has left important research and clinical achievements in the field of myasthenia gravis. He is deeply loved by patients. In his later years，he had long been committed to promoting the development of myasthenia gravis and the progress of public welfare undertakings. To express our deepest memory and gratitude，this report retains the unfinished preface by Professor Xu and the content of the three speeches at the report kick－off meeting. We would like to represent the Chinese patients with myasthenia gravis by expressing our deep gratitude and high respect for the painstaking efforts and contributions made by Professor Xu on clinical diagnosis，treatment and disease research!

Preface one

Summarize the past and look forward to the future

“Myasthenia gravis”（MG）is an autoimmune disease that affects acetylcholine receptors on the postsynaptic membranes at the junction of nerves and muscles and is characterized clinically by muscle weakness that is exacerbated by activity and relieved by rest. In China，the incidence rate is about 7.4 cases per million people per year，which classifies it as a rare disease. Women aged 18-35 and men aged 35-55 are high-risk groups. At present，there are about 650，000 patients with MG in China. When the condition is severe，the patient may need to use a ventilator to assist breathing due to the effect on chewing，swallowing and the breathing functions of the body，and the illness may even cause a "myasthenia gravis crisis" that is life-threatening.

Due to the lack of sufficient understanding of the disease by the public，some medical staff and patients，54. 15% of the patients had been misdiagnosed，36.68% of the patients experienced a myasthenia gravis crisis due to delays in the discovery and treatment，and some patients have even committed suicide due to lack of confidence in a cure for the disease.

A “2022 health report of patients with myasthenia gravis in China” investigated the living conditions of more than 1，200 patients with MG in China，and found many problems in their life，work and in the treatment of patients. We hope to bring attention to all aspects of society to make improvements at the medical level，reduce misdiagnosis and missed diagnosis，strengthen the publicity of science popularization，inform the public about MG，create a good social environment for the active rehabilitation of patients and greatly improve the quality of life of patients.

The following is compiled according to Professor Xu’s speech at the start-up meeting of the investigation on April 22，2022：

The health report of patients with MG in China is a systematic summary.

From the perspective of the future application of the report results，it also helps us look forward to the future and points out the direction we should go. In the future，we should work hard in this direction and work together to make improvements.

In addition，we should also systematically review the past，and look at what has been done in China in terms of research，laboratories and clinical treatment. I think it is also very important to guide further work in the future.

For example，in the early 1990s，we cooperated with a military hospital to study the therapeutic effects of steroid application，including its effectiveness and side effects. How effective was it？What were the side effects？What kind of reaction can be expected when start to use it？The impression was that if a large amount of steroids were used，it would be exacerbated within a week. If it is more than a week，it generally would not increase the exacerbation. These results are helpful to guide our clinical application of steroids.

In addition，we have learned prior lessons. There was a patient who was a doctor from Inner Mongolia. After he came，we told him that the steroids could be increased slowly，but if it was increased in small doses，it may also exacerbate the condition. He said that I am a doctor，I will go back and add it slowly，and I will contact you if there is any effect. His condition worsened when the small dose was increased from 5 mg to 50 mg. A phone call came，saying that Dr. Xu was dying，he couldn't come，his condition got worse，and he couldn't walk anymore. What this case tells us about the problem of adding steroids is that if you give it to many patients，you may feel that there is no exacerbation. In fact，the patient was discharged from the hospital after a small dose of a week or two. However，the patient went to other doctors after the exacerbation. But when the previous doctor reviewed the case, he concluded that it didn't exacerbate the patients.

So I think there are many things. In the future, we must systematically summarize the experience and lessons to guide better work in the future. This is very important. Today is not the time to talk about these specific methods and problems, but mainly to point out a direction for future efforts. Reviewing the past and summarizing lessons learned provides a better guide for future work and efforts. I think this is very important.

In addition, regarding the delay in diagnosis. I think it is mainly due to insufficient understanding. There are two levels. One is the level of patients, ordinary people. The second is that the medical staff are not very familiar with the disease, and when patients go see a doctor, the doctors do not know MG. The importance of these aspects is mentioned in international and national conferences every year, so it is very useful to do a lot of publicity work at the two levels of the general population and medical staff.

There is also the issue of recurrence. In the early 1990s, we worked with a military hospital to collaborate on research. Will steroids make the MG condition worse? After doing this (research), an article was published, and some reports were presented at conferences in North America. The general conclusion is that if you want to use steroids, you need to follow up for two years. That is, at the beginning of using steroids, if you use a large dose of 1,000 mg or even 2,000 mg per day, then use it for a week, and the conditions will get worse in a few days. Otherwise, there may be no exacerbation of the conditions within a week or later. This is the result of two years of follow-up study, not one week or two weeks. Additionally, some people say that small doses will not increase the severity. However，we have seen some people，who added 5 mg to 10 mg little by little without getting worse，but when the dose reached 50 mg or 60 mg，it did get worse. Therefore，the basis for not exacerbating with small doses is not enough. To draw such a conclusion，it would be better to have more factual evidence.

Lastly is the fertility issue. Typically，this issue attracts more attention for the female patients. However，is there an issue with male patients？The impact of the disease itself on the male patient，and the impact of the medication on the male patient. Will it have any adverse effects on the physiology？There is no factual basis for this.

For example，in the multiple sclerosis guidelines，which are legally binding，if you make a mistake according to the guide，there is no problem. If you do not follow this guideline，you are wrong，and you will be held legally responsible. Well，the guidelines for multiple sclerosis say that（Glatiramer Acetate）has no adverse effects on the pregnancy of multiple sclerosis patients. If the patient is pregnant during the treatment period，if she wants to change the drug，she can change it. If she is still using it during the treatment period，she can continue to use it again. The conclusion is that（this drug）is safe. But last year an American journal of ophthalmology sent an article and asked us to review it，so I reviewed it. The patient was a multiple sclerosis patient，and a deformity occurred with the patient's baby as a result of taking（drugs）. We suggested that he publish it. Why？Although the child is deformed，it cannot explain the causal relationship between the two，but the two are related in time factors. Then，as the previous guideline said，it is safe to use this drug. Therefore，it was recommended to publish this article to draw the public's attention to this issue in the future, not to say that it is a causal relationship. As a result, this article was published.

For MG, the research in this area may be even more lacking. For example, on the issue of vaccines, is there any benefit to getting the vaccine? For COVID-19, the advantage of vaccination is whether it can be prevented, and the disadvantage is whether MG can be exacerbated or relapsed. Who should answer this question? It should be the person who takes vaccine. If the side effects of this patient's relapse are not serious, it will play a great role in preventing future infection. Only by comparing the two aspects of gain and loss can we draw a conclusion.

I think we need more facts and actual materials, such as the contents of this report and the materials collected during follow-up. It's better to have a pragmatic attitude towards seeking truth from facts. I often tell my students that what a doctor sees is not a disease, but a patient. The more meticulous the observation and research, the better the patient will be.

So, summing up the past and looking forward to the future. Looking back, we have achieved a lot, and looking ahead, we will definitely achieve even better results, but more work needs to be done. That's all I have to say, and I will leave more precious time for the doctors who follow.

Former Chief Physician of Beijing Hospital of the Ministry of Health

Former Honorary Chairman of Beijing Aili Myasthenia

Gravis Rare Disease Care Center

Xu Xianhao

Preface two

Working Together to Promote Rare Disease Insurance Coverage

The 2022 Health Report of Patients with Myasthenia Gravis（MG）provides a wealth of information and offers a panoramic view of the patients’ living and health status，as well as their important experiences in the treatment process. This report，in particular，focuses on in-depth interviews with 33 patients and caregivers，providing a true-to-life extensive view of the patient journey of Chinese MG patients，including their current health conditions and basic societal needs.

The government places great emphasis on the treatment of patients with myasthenia gravis，especially how to protect them with medical services and insurance coverage. In recent years，most of the drugs we have developed for treating MG have been covered by national medical insurances. This not only ensures that MG patients have access to medication，but also ensures that medical expenses incurred are within their scope of affordability. It is evident from this report that medical insurance of MG patients is vital to their treatment and that it is an essential means of addressing their health issues.

However，I also believe that it is crucial to pay attention to the other aspects of the lives of patients with rare diseases，including those with MG. For example：

First， we must provide them with a safe and convenient living environment. We need to provide an environment that is convenient for them to travel around， such as through our public transportation systems and well-constructed road.

Secondly， we need to customize employment policies that ensure equitable access to job opportunities for these patients. Employment is crucial for people's livelihoods. Without it， patients will have no source of income， which can lead to poverty for their families and a diminished quality of life. Therefore， we must address their employment issues in a fair and equitable manner

Thirdly， we should work towards gradually implementing a long-term care insurance system， as many MG patients require partial or full-time care， which places a significant financial strain on both themselves and their families.

Therefore， I call on the entire society to pay attention to the living conditions and disease treatment of all rare disease patients， including those with MG.

Regarding this report， I have several suggestions. Firstly， in the current report， some data provide us with goodknowledge of the employment and education status of MG patients. However， there is a lack of comparative data between them and other groups of people. This results in a limited understanding of their current static circumstances， as their situation is not compared to that of the general public to determine the extent of the disparity.

Secondly， for the many problems discovered， we need to conduct some analysis for their social causes. When reading this report， I paid close attention to the medical insurance for MG drugs. I noticed that all drugs were included in medical insurance，but many patients reported not receiving reimbursement. We need to examine the underlying reasons for this，including whether their medical expenses fell below the coverage limit of their insurance，or if they only received outpatient care，as some regions only reimburse hospitalization costs and other related factors. Therefore，I suggest that we conduct an extensive analysis of their causes. Only through this analysis can we discover problems and provide policy recommendations to relevant government departments.

Another issue is that many patients have not participated in any schemes of medical insurance. What are the reasons for this？Is it because our policy promotion is not strong enough，or because they cannot afford to pay for the insurance？We need to explore the reasons behind this.

Overall，this report is a rare and valuable document. I hope that it will attract the attention of the entire society and provide relevant departments with information for references when formulating policies. Furthermore，I hope that the data from this report can be utilized in academic research.

Let us work together to promote the support work for rare diseases patients，includingthose with MG. We should also actively promote legislation for them，so that rare disease patients，including those with MG，can live in a fair and just social environment.

Xiong Xianjun
Former director of the Medical Services Management Division of the National Healthcare Security Administration（Chiefs at the department and bureau level）

Foreword

February 2023 will be a memorable month. After three years of pandemic prevention and control，life has gradually returned to normal，and Aili Myasthenia Gravis Care Center is also celebrating its tenth anniversary.

Over the past ten years，Aili Care Center has completed four large-scale research studies on myasthenia gravis（MG）patients in China，which have contributed significantly to public welfare promotion and scientific research development. A variety of channels have been used to disseminate the content of the reports，including traditional media，self-media，print and electronic publications，among others. A total of 4，000 copies of the reports were distributed one-to-one，and the reports were also shared in hundreds of sharing activities. The number of valid questionnaires in 2014 was 614；in 2018，it was 1，896；in 2019，it was 2，397 and in this study，there were 1，020. In addition，33 in-depth interviews lasting between two to five hours were conducted with patients and their families，and seven interviews were conducted with physicians and medical practitioners. While the 5，967 questionnaires and interviews reflected the changes in our research work and the social environment，what remains unchanged is the hope of the patients for a better life and their desire for a cure.

A journey of a thousand miles begins with a single step.

During the early days of Aili's establishment，when we published information on the conditions of MG patients in various venues，we hoped to attract media attention by mentioning the difficult patient journey，unmet needs for medication，lack of social insurance and poor living conditions. However，when people，the government，pharmaceutical companies and other parties paid attention，apart from the sad stories of patients struggling to live a better life，the questions often asked were：Do we have any data supporting our argument？What is our sampling size？How often do crises take place？How common are these plights？What is the average annual cost of treatment for a patient？Brother Hailian，another founder，and I have always had difficulty responding to these questions，which led to our determination to conduct research about patients’ living conditions.

In 2014，the Health Report of Patients with Myasthenia Gravis in China was released for the first time. Patients ranged in age from the youngest，a 4-year-old child，to the oldest，an 80-year-old elderly person. The results of the study validated our subjective observations in the field. The report provided Aili the opportunity to co-launch the 6-15 Myasthenia Gravis Care Day with several other parties that year，which presented for the first time the living conditions of MG patients in China. A number of patients provided the report to their local medical insurance department and successfully secured outpatient reimbursement for MG.

Following this，in 2018，our report entitled Myasthenia Gravis Patient Survival Report-A Comparative Perspective from China and the United States was released. The large gap in the quality of life between Chinese and American patients reflected the more challenging situation faced by Chinese patients，and the difference in drug use reflected a gap in patient awareness as well as medical care. It was clear from the report that patient living conditions were still difficult and that there were vast unmet needs. Moreover，the 2018 study examined，for the first time，the impact of comorbidities on MG patients，as well as their emotional psychology，quality of life assessment and gender differences.

Also in 2018，multiple national ministries and commissions jointly released the List of Rare Diseases in China，and MG was listed among them. In taking advantage of this opportunity，MG patients' living conditions，unmet needs and Aili's work received unprecedented attention. In addition，one after another，news of new drug research and development from across the globe have been announced.

This was the experience of Aili in the first five years of its establishment. A wide range of advocacy and service work has been conducted around improving the living conditions of patients，including the advocacy of scientific knowledge，patient assistance and education，emotional support，community empowerment and social integration. A new light has been shed on the small effort that was initially unseen，and it has made us feel differently-it can actually be seen，and a real change is taking place.

In the five years since then，various policy initiatives and media coverage have created a push for the development of rare disease treatments. Myasthenia gravis has been a part of that benefit and a part of the driving force.

In terms of each individual study，data collection and analysis have been meticulously conducted. From a chronological perspective，Aili's series of surveys can be seen as part of a larger narrative.

Today，ten years later，the report shows a significant reduction in the time to diagnosis，a decrease in the incidence of critical illness and ventilator use，an increase in the choice of drugs available to patients，a rise in awareness and an improvement in their conditions. Additionally，there has also been a shift from purely survival issues to the need for better and safer drug needs and disease management, and a gradual change of focus from local outpatient coverage to multi-payer reimbursement for new drugs.

This is why our study this year shifted the focus from the patients' survival to the more general health conditions. Through 33 in-depth interviews, the first ever Myasthenia Gravis Patient Journey in China has been developed, supplemented by questionnaire data, to provide a realistic picture of the problems encountered at each stage of the patient's journey. These problems include the patients' medical consultation and disease management, their medical compliance, the perspectives from both the patient and their families, as well as their relapse and disease progression.

Seeking treatment for MG is a long, winding and undulating journey for patients.

As part of this endeavor, we hope to examine the macro picture of MG patients in China from a temporal perspective, to look at nuanced differences in each individuals' stories and to explore deeply the effect of the disease on the health of patients, in which both the group and individual are equally important. In the post-epidemic era, we hope to offer a forum for reflection for all parties, providing the government, hospitals, medical experts, pharmaceutical companies and service providers with a deeper understanding of the difficulties faced by MG patients. This may serve as the starting point for the complete cure of the disease.

Many people are involved in the release of the report, and their hearts and souls are at stake. On behalf of Aili and the patients, I would like to express our deep gratitude for the efforts and support of all parties.

Sadly, Professor Xu Xianhao, the medical advisor to the current study, Honorary Chairman of the Board of Directors of Aili Care Centre，and a leading neuroimmunologist who had been supporting Aili for the past decade，passed away during the writing of this report.

We would also like to thank our other medical advisor，Professor Yang Huan，Chief Physician of the Department of Neurology at Xiangya Hospital. As our study coincided with the outbreak of the pandemic，Doctor Yang took on responsibility for the whole community，dedicating a considerable amount of time and energy to supporting research. In addition，we would like to thank the seven specialists on Myasthenia Gravis who participated in our interviews and provided valuable insights from the perspectives of both biomedicine and Chinese traditional medicine. In order to protect the information of the interviewees，no public acknowledgments will be made here.

This research is supported by the China Red Cross Foundation. As the only rare disease report in the "Red Book" report series，this report is expected to be seen and valued by more relevant parties.

Professor Dong Dong，Board Member of Aili Care Centre，Associate Researcher of Shenzhen Research Institute of the Chinese University of Hong Kong and Head of the Real World Data Lab for Rare Diseases，led the team to conduct extensive research and analysis，which resulted in many new concerns being identified. Having focused on the studies of MG since 2015，she has led the execution of several research studies related to MG and has published many relevant research findings.

Moreover，I would like to thank the 1，053 patients and families who provided information，Janssen China for their vital support，all of the members of the research team，the full-time and part-time partners of the Aili Care Center，volunteers and patient care workers for their dedication and commitment!

Our special gratitude also goes to Professor Zhao Chongbo of Huashan Hospital, Professor Li Haifeng of Xuanwu Hospital, and Professor Yin Jian of Beijing Hospital, who have long supported Aili's research efforts.

MG patients are like snowflakes. No one is the same, but their suffering can be melted by warmth. There is a unique life behind every piece of data, which deserves to be seen and respected. Everyone has the right to life and medical treatment, as well as the right to enjoy health and happiness.

May we no longer hear sad stories. Let the powerless be powerful, let the powerful move forward, and let the world be bathed in love and warmth!

Beijing Aili Myasthenia Gravis Rare Disease Care Center
Founder/Director: Qingzhao
February 2023

CONTENTS

Part Ⅰ

Research Origin and Research Introduction

1. About the Survey

1）Disease background and research purpose

Myasthenia gravis（MG）is anacquired autoimmune disorder，mediated by autoantibodies，surrounding the neurotransmission at the neuromuscular junction（NMJ）.

Symptomsof MG include varying degrees of muscle weakness，which often affects the eyes，face，and muscles associated with swallowing，and may cause diplopia（i.e.，double vision），drooping eyelids，speech difficulties，and walking difficulties. Antibodies to the acetylcholine receptor（AChR）are the most common pathogenic antibodies. In addition，other components which target the postsynaptic membrane，including muscle-specific receptor tyrosine kinase（MuSK），low-density lipoprotein receptor-related protein 4（LRP4）and ryanodine receptor（RyR）antibodies have been found to be involved in the pathogenesis of MG. These antibodies can interfere with AChR aggregation，affecting AChR's functioning and NMJ signaling.

The global prevalence of MG is 150-250 per million，and the estimated annual incidence is 4-10 per million. The incidence of MG in China is 0.68 per one-hundred thousand，with the incidence among females being slightly higher. The in-hospital mortality rate is 14.69%，with the main causes of death being respiratory failure and pulmonary infection. The disease can occur at all ages，but occurs most frequently between the ages of 30 to 50. The incidence of JMG（juvenile myasthenia gravis）among Chinese children and adolescents is as high as 50%，which constitutes another peak of incidence. The latest epidemiological survey shows that the 70-74 year old age group is a high-risk group in China.

At present，the treatment of MG mainly includes cholinesterase inhibitors，glucocorticoids，immunosuppressants，intravenous immunoglobulins（IVIG），plasma exchange（PE）and thymectomy. The medical community has also made important progress in monoclonal antibodies and biological targeted drugs. To promote the public and government understanding of MG and improve the quality of life of patients with MG，this study aims to investigate the daily life of patients with MG，couched in terms of their patient journey，medical burden，social support，and unmet needs.

2）Study Design

This study adopted a sequential mixed methods study design. This study explores the living condition of patients with MG in China by combining qualitative research methods surrounding in-depth interviews and quantitative research methods on cross-sectional surveys.

The qualitativeinterview officially launched in January 2022 and ended in May 2022. In January 2022，the recruitment for patients as interview subjects began. More than 70 patients and their family caregivers registered for the study，patients and their families who met the inclusion criteria were selected. The interview method was semi-structured in-depth interview. Each patient or family member interview lasted 3-5 hours. The patient interview guide mainly consisted of background situation，patient journey（onset，diagnosis，going to the hospital，treatment，daily management of the disease，etc.），psychological and daily-life support，and others（such as economic burden，employment，etc.）. In April，interviews with doctors as participants began. The research team interviewed a total of 7 doctors who were from neurology，thoracic surgery，and traditional Chinese medicine specialties/departments. Some of the doctors interviewed were found to have a close relationship with some of the interviewed MG patients and were mentioned by many of them. Regarding the patient journey and medical experience from the patient interview，we formulated a doctor interview guide based on the department of the interviewed doctor. The interviews for each doctor lasted around 1-2 hours. The main contents of the interview include background information of the doctor，diagnosis and treatment experience related to MG，understanding of diagnosis，treatment and rehabilitation in different departments，and others（fertility，refractory，doctor and patient communication，traditional Chinese medicine，psychology，foreign experience，etc.）. As of 14 May，2022，a total of 33 interviews with patients or their family members were completed，consisting of 26 patients and 7 family members. Among the family members interviewed，3 were family members of the interviewed MG patients of this study，and the other 4 were not. Therefore，the number of interviewees regarding patient journey is 30. In addition，the study also completed 7 doctor interviews，consisting of 3 neurologists，2 thoracic surgeons，and 2 Chinese medicine practitioners.

The quantitativesurvey officially launched on 6 June，2022 and ended on 13 July，2022. A total of 1，020 valid questionnaires were collected through a nationwide online survey. Of the valid questionnaires，a total of 935 questionnaires were filled out by patients themselves，and 85 were filled out by their family members or other primary caregivers on behalf of the patient. The questionnaire collected the patients' sociodemographic information，lifestyle，disease characteristics，medical treatment experience，medical expenditure，quality of life and relevant data，as well as the main caregivers' information on social support and impairement on working productivity and daily activty.

3）Research Ethics

This study hasobtained ethical approval from the Survey and Behavioral Research Ethics Committee of the Chinese University of Hong Kong（Review ID：SBRE-21-0260）. All patients/family members and doctors who participated in the in-depth interviews and questionnaires underwent a detailed informed consent procedure，and were fully informed about the significance，risks and benefits of the interviews and questionnaires. The interviews and questionnaires began only after informed consent was obtained. All interviews were transcribed in verbatim，and all participants in the final report were anonymized.

4）Data Analysis

Data analysis for qualitativestudy was performed in two iterative phases. In the first phase，two researchers jointly hand-coded ten interview transcripts to identify recurring themes by comparing similar or related sections between the transcripts. After the first stage of manual coding，seven main stages of the MG patient journey were identified：（ⅰ）Onset；（ⅱ）Diagnosis；（ⅲ）Treatment；（iv）Relapse and Crisis；（v）Patient Organization Support；（vi）Recovery；and（vii）Current medical condition and other needs. Furthermore，sub-themes that emerged under each major stage were identified. In the second stage of data analysis，the qualitative analysis software Dedoose was used to code and analyze all the interview transcripts，continuously update，and improve the classification of sub-themes. After the coding was completed，the results of the analysis were exchanged and discussed with the research team.

For the quantitative research，different variables were statistically presented，categorical variables（such as gender，age，hukou，etc.）were presented as count（percetage），and continuous variables（such as income，medical expenditure，quality of life scale scores，etc.）were described by mean （ SD ）， median， minimum， maximum， according to the variable charcteristics. All statistical analyses were conduced using the statistical software Stata 16.0.

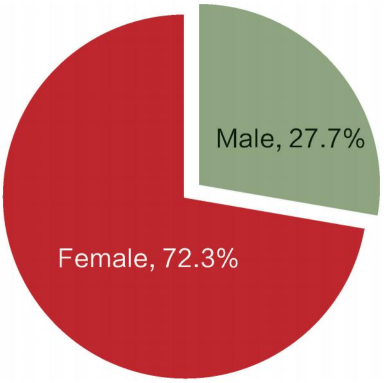
Part Ⅱ

Investigation on the Living Condition of Myasthenia Gravis Patients

2. Demographic information

1）Gender

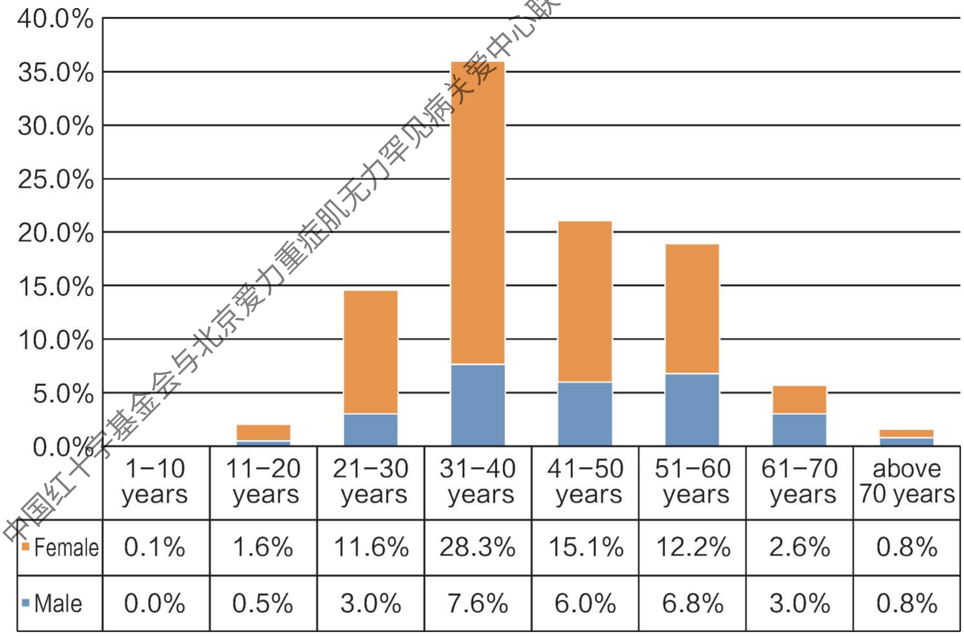
Among the 1020 respondents, there were 283 male patients, accounting for 27.7% of the study population, and 737 female patients, accounting for 72.3%.



2）Age

Among the respondents，the 31-40 year old age group was the largest，with a total of 367 people，accounting for 35.9%；followed by the 41-50 year old age group，with a total of 215 patients，accounting for 21.1%；the 1-10 year old age group was the smallest with only 1 person，accounting for 0.1%. The average age of the patients was 41.7 years，and the median age was 39 years.

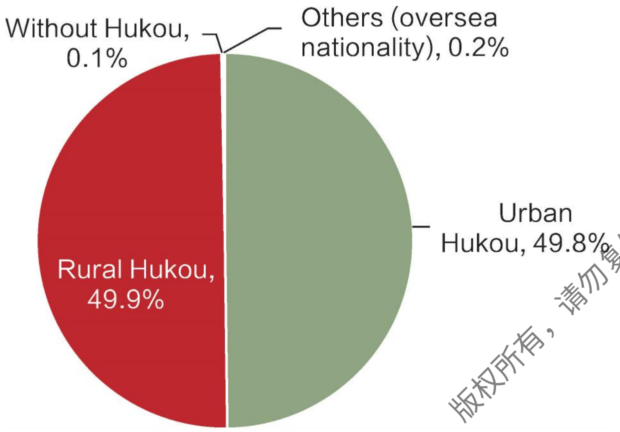
When comparing males to females，the proportions of males and females in the age groups of 1-10 years old，61-70 years old，and over 70 years old were largely equal，and the proportion of females in other age groups was higher than that of males.



	1–10 years	11–20 years	21–30 years	31–40 years	41–50 years	51–60 years	61–70 years	above 70 years
▪ Female	0.1%	1.6%	11.6%	28.3%	15.1%	12.2%	2.6%	0.8%
▪ Male	0.0%	0.5%	3.0%	7.6%	6.0%	6.8%	3.0%	0.8%

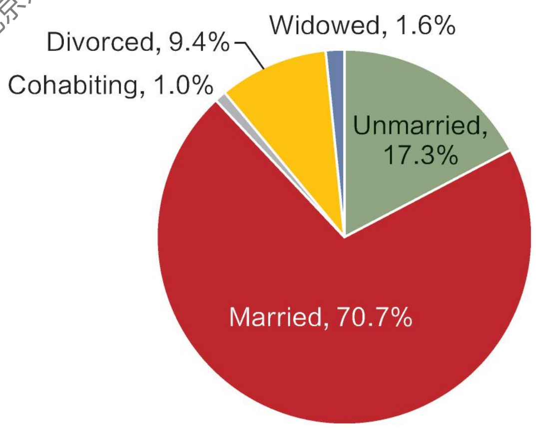
3）Hukou

Among the respondents，therewere 509 people with rural Hukou， accounting for 49.9%；508 people with urban/non-rural Hukou，accounting for 49.8%；2 people with overseas nationality，accounting for 0.2%；and 1 person without hukou，accounting for 0.1%.



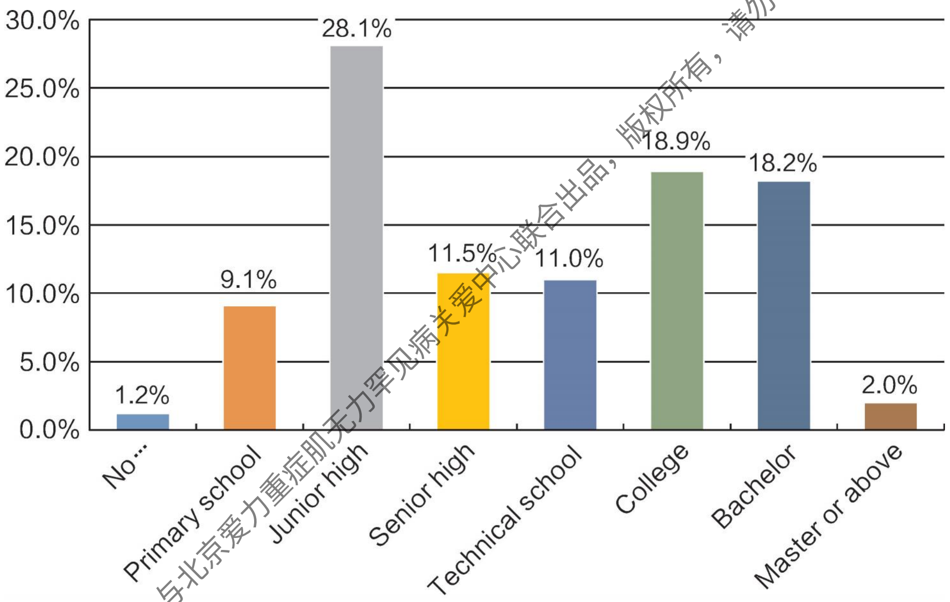
4）Marital status

Among the adult respondents，the number of married peoplewas the largest，with a total of 714 people，accounting for 70.7%；175 people were unmarried，accounting for 17.3%；and the number of cohabiting people was the smallest，with a total of 10 people，accounting for 1.0%.



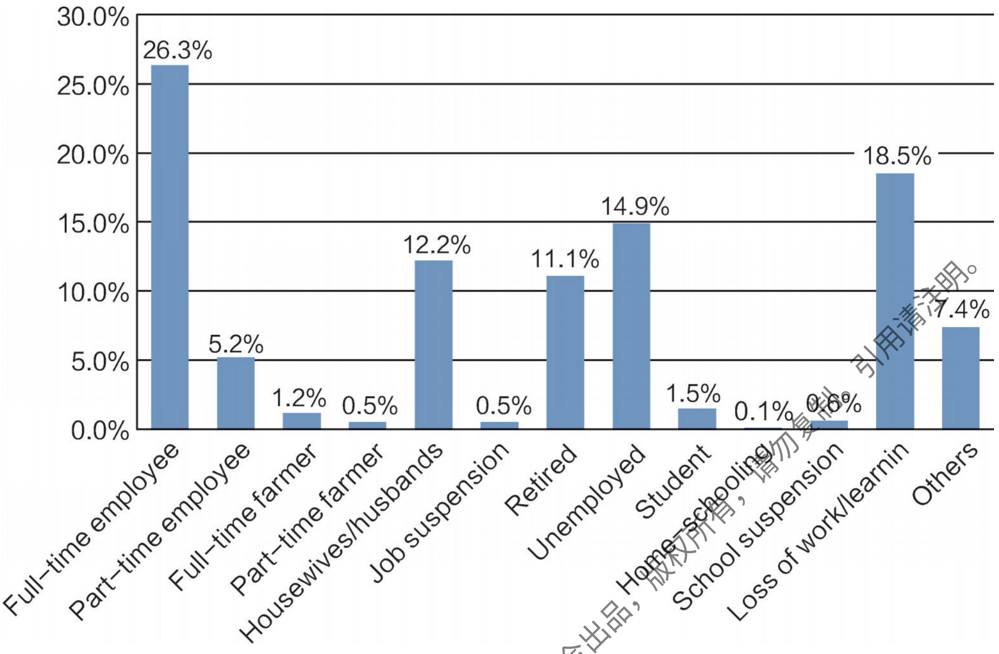
5）Education level

Among the respondents，the number of junior high school graduateswas the largest，with a total of 287 people，accounting for 28.1%；the second largest group was the college graduates，with a total of 193 people，accounting for 18.9%；and the number of people with no school/preschool education level was the least，with a total of 12 people，accounting for 1.2%. Overall，the proportion of people with higher education was 39.1%.

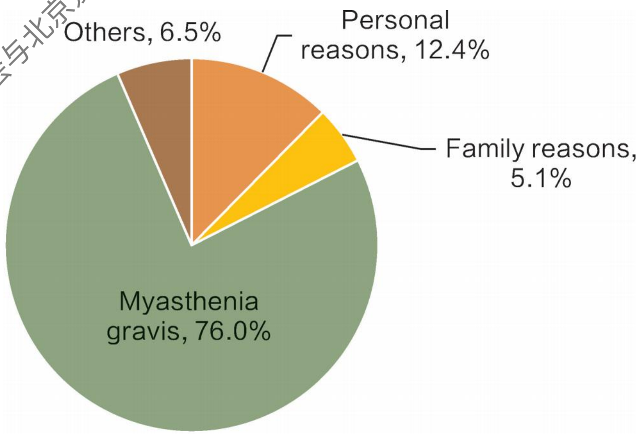


6）Employment status

Among the 1，011 adult respondents，the number of full-time employeeswas the largest，with a total of 266 people，accounting for 26.3%；this was followed by those with loss of labor/learning ability，with a total of 187 people，accounting for 18.5%.



Among the 1，011 adult respondents，612 werenot full-time employed/studying/farming，accounting for 60.5%. Among this group，the most common cause of the current employment/academic status was MG，with a total of 465 people，accounting for 76.0%；this was followed by personal reasons/personal choice，with a total of 76 people，accounting for 12.4%.



7）Insurance/security status

Among the respondents，the number of people who enjoy basic medical insurance for urban employeeswas the largest，with a total of 371 people，accounting for 36.4%；followed by the new rural cooperative medical care，a total of 149 people，accounting for 14.6%；and 118 people did not have any health medical insurance，accounting for 11.6%.

Current Health Insurance	Numbers	%
Medical Insurance for Urban Employees	371	36.4%
New Rural Cooperative Medical Care	293	28.7%
Basic Medical Care for Urban Residents	149	14.6%
Free Medical Care	80	7.8%
Supplementary Medical Care	66	6.5%
Cooperative Medical Care for Urban & Rural Residents	64	6.3%
Commercial Health Insurance	54	5.3%

Current Health Insurance	Numbers	%
Others	13	1.3%
None of Above	118	11.6%

A total of 11.2% of the respondentswere receiving medical charity assistance，of which 42 people had received help from private rare disease assistance funds，accounting for 4.1%；and 42 people were the recipients of government medical assistance，accounting for 4.1%.

Current Charity Medical Help Project	Numbers	%
Private Medical Aids for Rare Diseases	42	4.1%
Government Medical Assistance	42	4.1%
Online Donation	22	2.2%
Others	16	1.6%
None of Above	906	88.8%

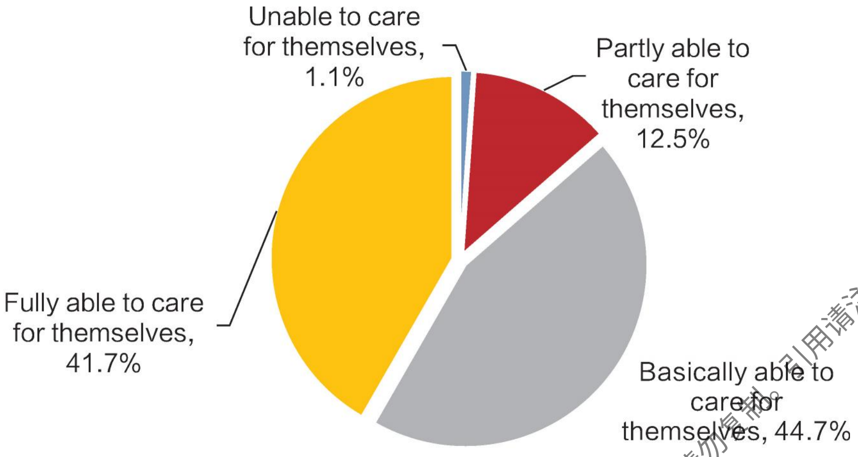
A total of 64.8% of the respondentsreceived social security/insurance，of which the largest number of people received endowment insurance，with a total of 350 people，accounting for 34.3%；followed by the minimum living security， with a total of 169 people，accounting for 16.6%；and 108 people received security for the disabled，accounting for 10.6%.

Current Security/Insurance	Number	%
Endowment Insurance	350	34.3%
Minimum Living Security	169	16.6%
Security for the Disabled	108	10.6%
Other Social Security	34	3.3%
None of Above	394	38.6%

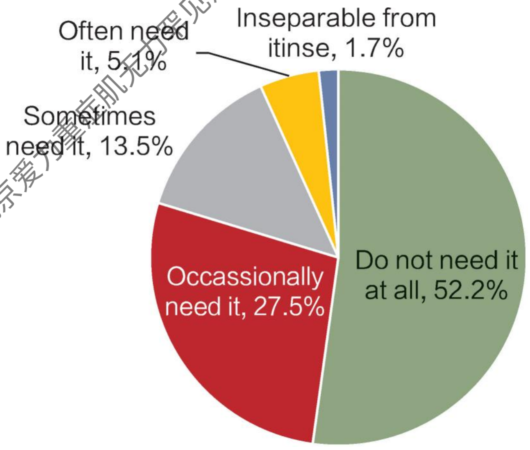
3. Physical function and comorbidities

1）Self-care ability

Among the respondents，the number of patients who can basically take care of themselveswas the largest，with a total of 456 patients，accounting for 44.7%；followed by patients who can fully take care of themselves，with 425 patients，accounting for 41.7%；a total of 128 patients required relatively more assistance，accounting for 12.5%. The number of patients who were completely unable to take care of themselves was the smallest，with only 11 patients，accounting for 1.1%.

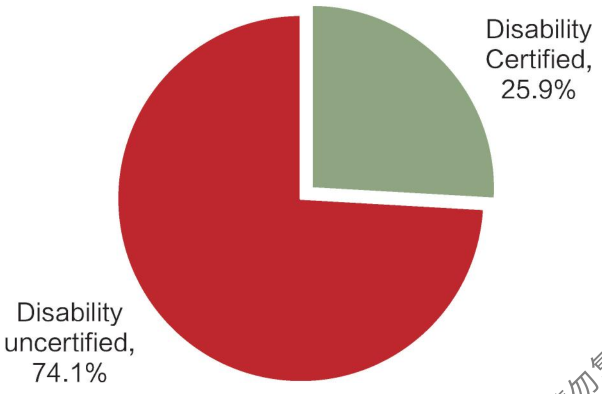


A total of 488 patients needed to use assistive devices in daily life，accounting for 47. 8%； the number of patients who occasionally needed assistive devices was the largest，with 281 patients，accounting for 27.5%；the number of patients who were completely inseparable from assistive devices was thesmallest，with a total of 17 people，accounting for 1.7%.

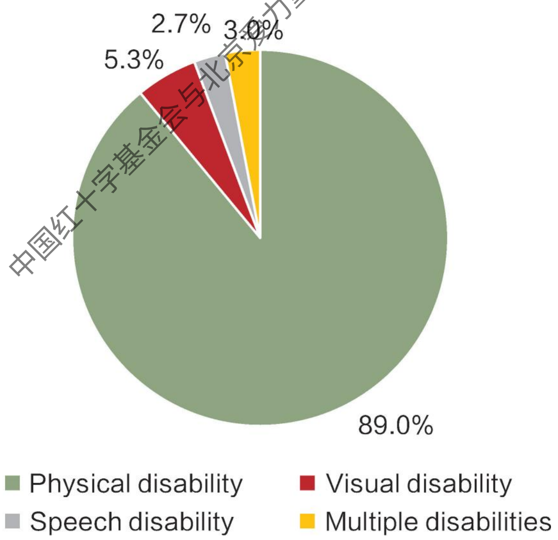


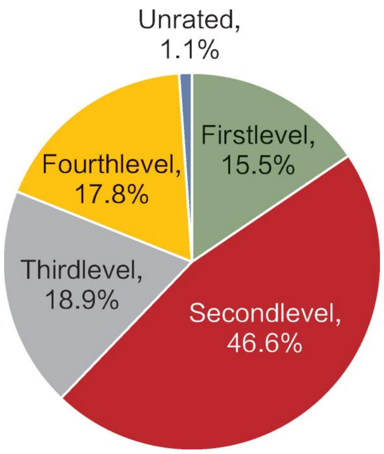
2）Registration for Disability Certificate

Among therespondents，264 people had applied for a disability certificate，accounting for 25. 9%； 756 people had not yet applied for a disability certificate，accounting for 74.1%.

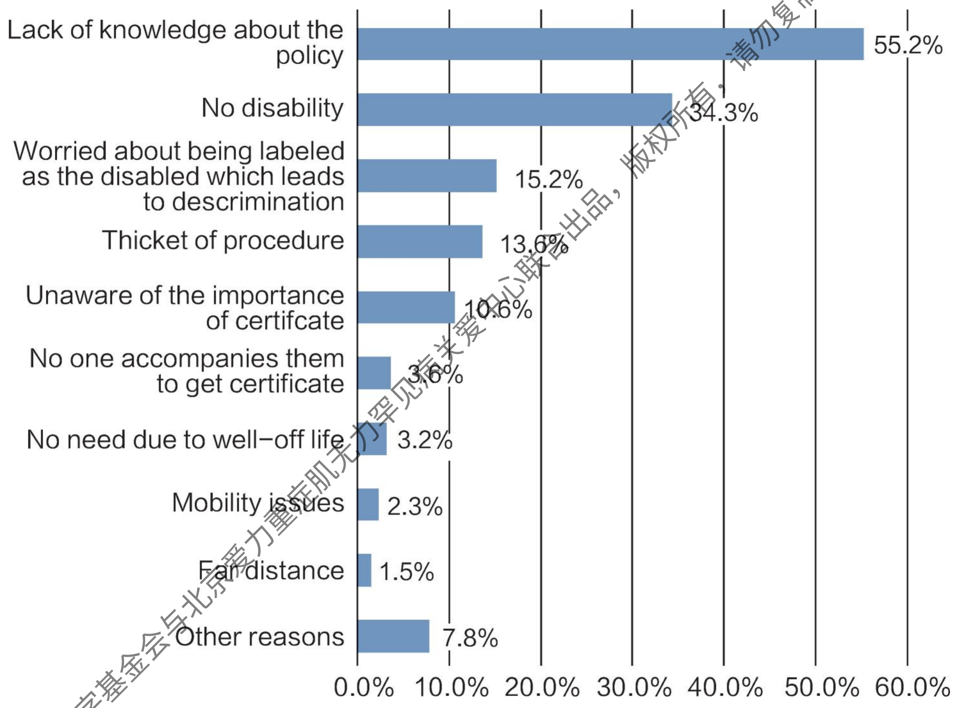


Among the 264 patients who had obtained the disability certificate，the number of people who have obtained the disability certificate of the physical disability was the largest，with a total of 235 patients，accounting for 89.0%；followed by the visual disability，with a total of 14 patients，accounting for 5.3%；the majority of patients obtained certificate of second-level disability，with a total of 123 people，accounting for 46.6%；this was followed by third-level disability，with a total of 50 people，accounting for 18.9%.



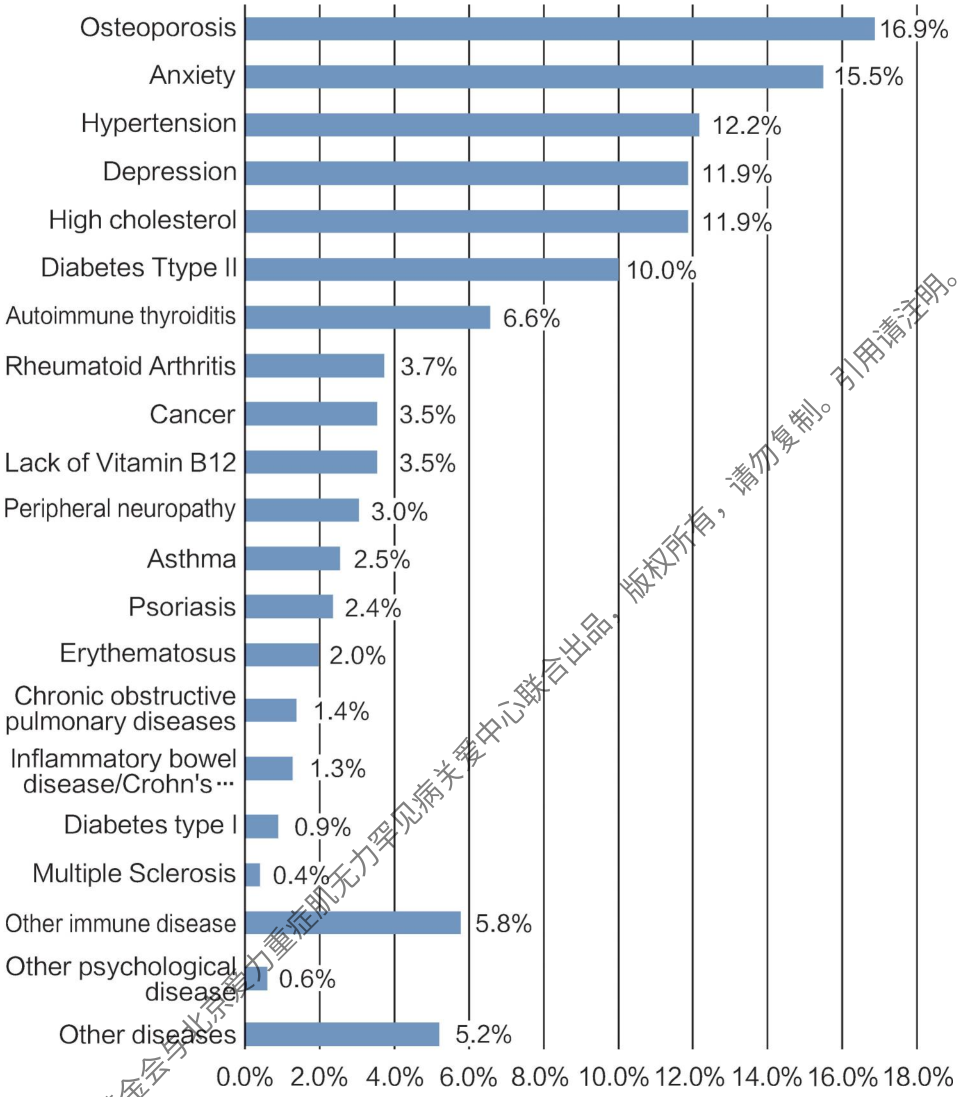


Among the 756 patients who had not yet applied for a disability certificate，417 patients did not understand the policy and did not know what to do，accounting for 55.2%；followed by 259 people who did not apply because they didn't have the need，accounting for 34.3%；the number of patients who did not apply because they lived too far was the least，with only 11 people，accounting for 1.5%.



3）Comorbidities

Among therespondents，a total of 172 patients with MG also suffered from osteoporosis，accounting for 16.9%；this was followed by patients suffering from anxiety disorders，with a total of 158 patients，accounting for 15.5%.

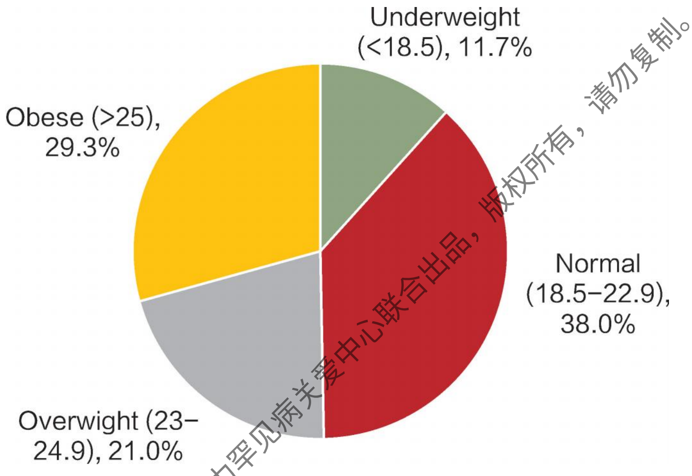


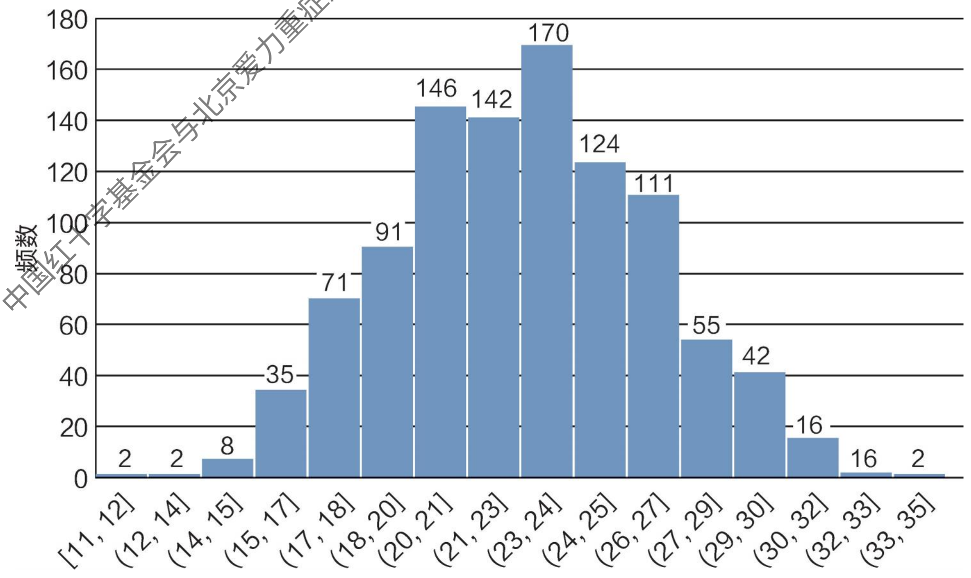
4. Lifestyle

1）BMI

BodyMass Index（BMI）is a commonly used international standard to measure obesity，the calculated index can be used to determine whether it falls within a “normal” range. The formula for calculating BMI is：BMI＝weight ÷ height^2 .

According to the classification of BMI in the Asia-Pacific region by WHO，among 1011 adultrespondents，most patients’ BMI was within the normal range（BMI 18.5-22.9），with a total of 385 patients，accounting for 38.0%；299 patients were obese（BMI>25），accounting for 29.3%；214 patients were overweight（BMI 23-24.9），accounting for 21.0%；and underweight（BMI＜18.5）patients had the smallest proportion，with 119 patients，accounting for 11.7%.



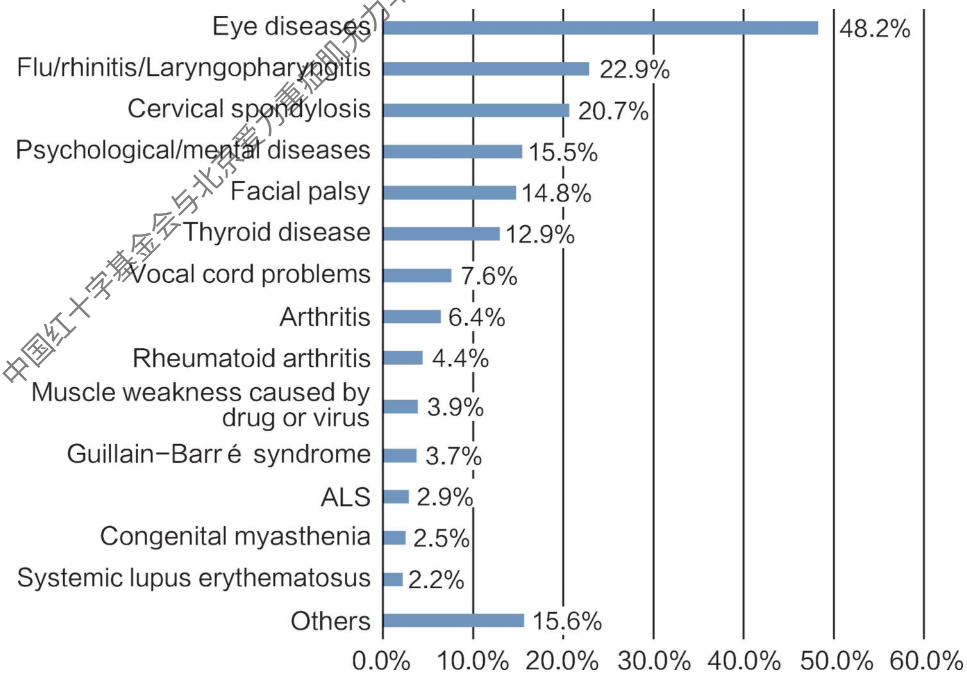


2）Diet

For leafygreen vegetables intake，most patients took it with the frequency of once a day，with a total of 283 patients，accounting for 27.7%；followed by patients who took 2-3 times a week，with a total of 234 patients，accounting for 22.9%；and 31 patients took it once a week，accounting for 3.0%.

For fruit intake，theintake frequency of 2-3 times a week had the highest number of patients，with a total of 327 patients，accounting for 32.1%；followed by patients who took fruit once a day，with a total of 205 patients，accounting for 20.1%；and 125 patients took fruit less than once a week，accounting for 12.3%.

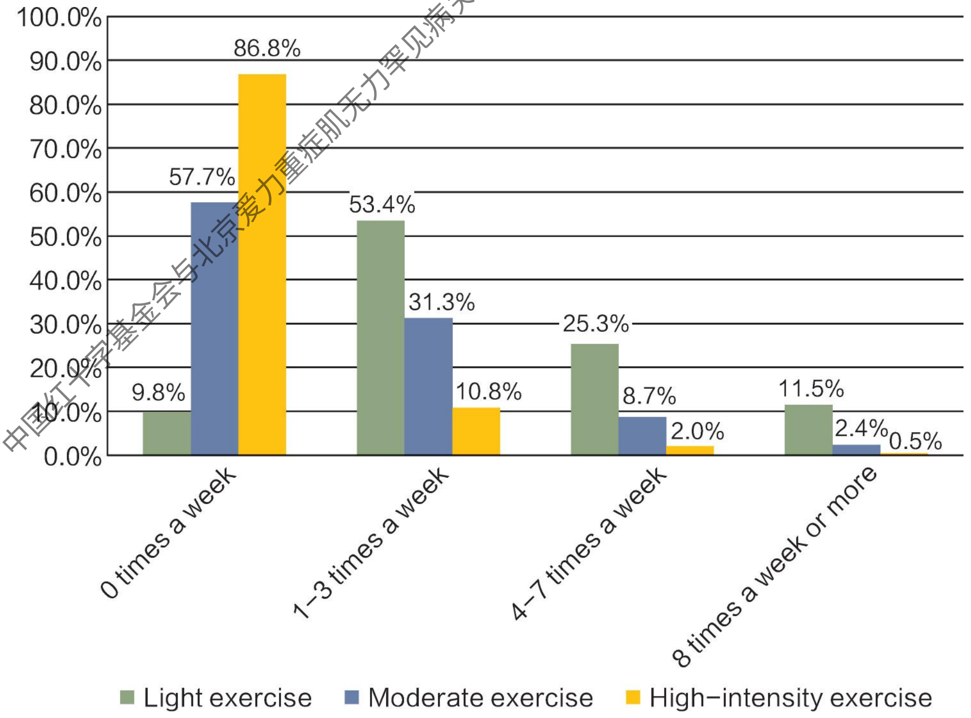
For high-fiber cereal intake，the number of patients withintake frequency of less than once a week was the highest，with a total of 360 patients，accounting for 35.3%；this was followed by patients with 2-3 intakes a week，with a total of 237 patients，accounting for 23.2%.



3）Exercise

Exercise in everyday life comes in many different forms. Generally speaking，light exercise includes light housework（such as cleaning，dusting），walking，visiting shopping malls，etc.；moderate daily exercise includes brisk walking，cycling，gardening，dancing，and Tai Chi，etc.；high-intensity exercise includes running，aerobic exercise，farming，strenuous ball games such as football and basketball，etc.

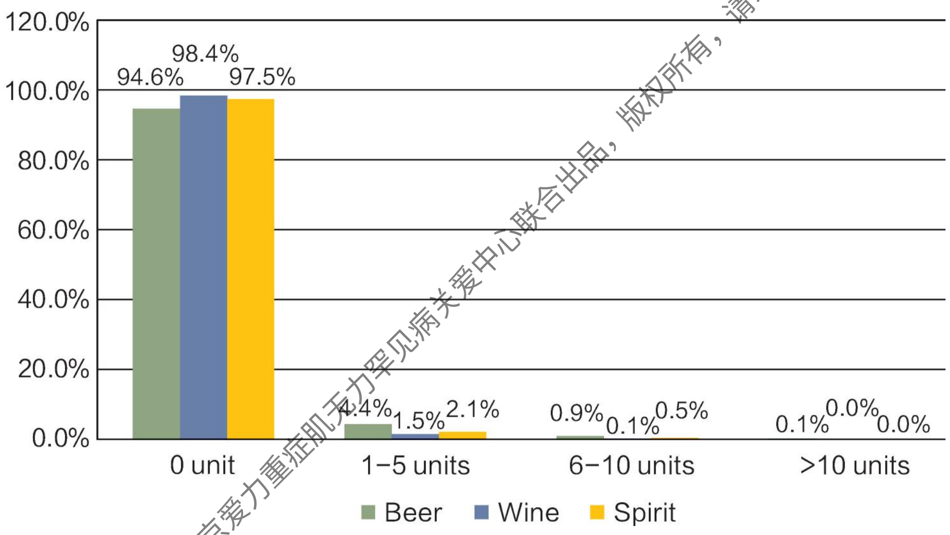
In this survey，it was found thatthe vast majority of MG patients did not perform high-intensity exercise，and only 110 patients（10.8%）performed high-intensity exercise 1-3 times a week. Most patients performed light exercise every week，accounting for 90.2%，and the number of patients who performed mild exercise 1-3 times a week was the largest，with a total of 545 patients，accounting for 53.4%. About 42.4% of the patients did moderate-



intensity exercise every week，and 31.1% of patients did moderate-intensity exercise 1-3 times a week，with a total of 317 patients.

4）Alcohol use

Amongthe respondents，the proportion of patients drinking beer，wine and spirits was 5.4%，1.6% and 2.6% respectively. A total of 4.4% of patients drank 1-5 units of beer a week，1.5% of patients drank 1-5 units of wine a week，and 2.1% of patients drank 1-5 units of spirits a week.①



5）Smoking

Among therespondents，92 were current smokers，accounting for 9.0%. Among the 928 non-smoker patients，825 had never smoked，accounting for 88.9%；103 had quit smoking，accounting for 11.1%.

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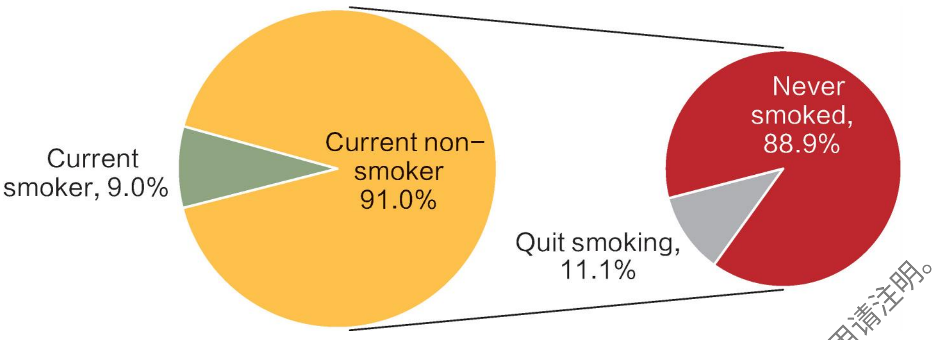
① Note：Unit beer：about 284～340ml，one can of 330ml small-canned beer
Unit wine：about 85～142ml，125ml/1 goblet of red wine
Unit spirits：about 28～43ml，half taels/to one tael of white wine，such as half a bottle of Xiaoerguotou in 22 packs



5. Diagnosis and examination

1）MGFA classification

According to the “Guidelines for the Diagnosis and Treatment of Myasthenia Gravis in China（2020 Edition）”，for the classification of MG patients，it is recommended to use the method jointly proposed by the American Academy of Neurology and the Myasthenia Gravis Foundation of America（MGFA，Myasthenia Gravis Foundation of America）for identification and classification. The MGFA classification is designed to quantitatively assess the severity of the disease in MG patients but is not intended to be used for efficacy and prognostic evaluation.

The MGFA classification is as follows：

Type	Clinical Symptoms
Ⅰ Type Ⅰ	Any ocular muscle weakness. May have weakness of eye closure. All other muscle strength is normal.

Type	Clinical Symptoms
Ⅱ Type Ⅱ	Mild weakness affecting muscles other than ocular muscles. May also have ocular muscle weakness of any severity.
Ⅱa Type Ⅱa	Predominantly affecting limb or axial muscles or both. May also have lesser involvement of oropharyngeal muscles.
Ⅱb Type Ⅱb	Predominantly affecting oropharyngeal or respiratory muscles. May also have lesser or equal involvement of limb or axial muscles.
Ⅲ Type Ⅲ	Moderate weakness affecting other than ocular muscles. May also have ocular muscle weakness of any severity.
Ⅲa Type Ⅲa	Predominantly affecting limb or axial muscles or both. May also have lesser involvement of oropharyngeal muscles.
Ⅲb Type Ⅲb	Predominantly affecting oropharyngeal or respiratory muscles. May also have lesser or equal involvement of limb or axial muscles.
Ⅳ Type Ⅳ	Severe weakness affecting other than ocular muscles. May also have ocular muscle weakness of any severity.

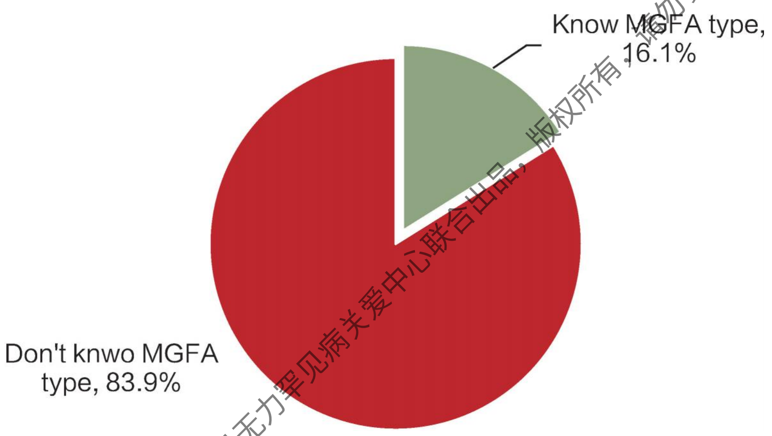
Type	Clinical Symptoms
Ⅳa Type Ⅳa	Predominantly affecting limb and/or axial muscles. May also have lesser or equal involvement of oropharyngeal muscles.
Ⅳb Type Ⅳb	Predominantly affecting oropharyngeal or respiratory muscles or both. May also have lesser or equal involvement of limb or axial muscles or both.
Ⅴ Type Ⅴ	Defined by intubation，with or without mechanical ventilation，except when employed during routine postoperative management. The use of a feeding tube without intubation places the patient in class Ⅳb.

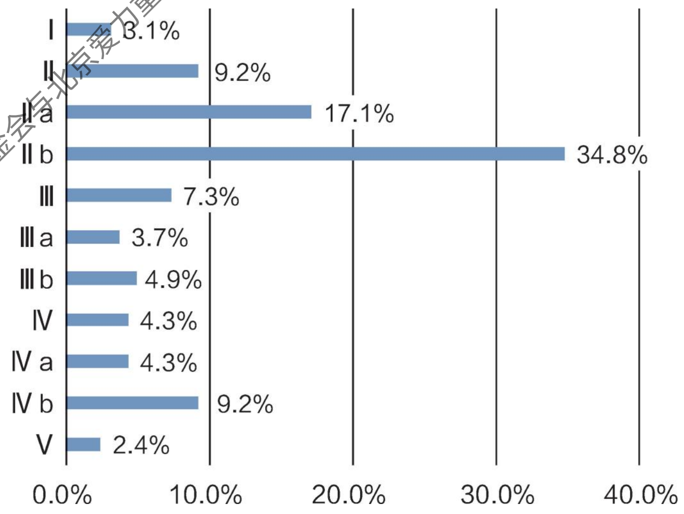
Among therespondents，164 people knew their MGFA clinical classification，accounting for 16.1%；856 people did not know their MGFA clinical classification，accounting for 83.9%.

Among the 164 patients who knew their MGFAclassification，some patients did not know which subtype they belonged to，so they could only provide the main category of clinical classification，such as type II，type III or type IV. Among the patients who knew their subtypes，type IIb had the largest number of patients，with 57 patients，accounting for 34.8%；followed by type IIa，with 28 patients，accounting for 17.1%. Among all the patients who knew their main category，the number of patients with type V was the least，with a total of 4 patients，accounting for 2.4%

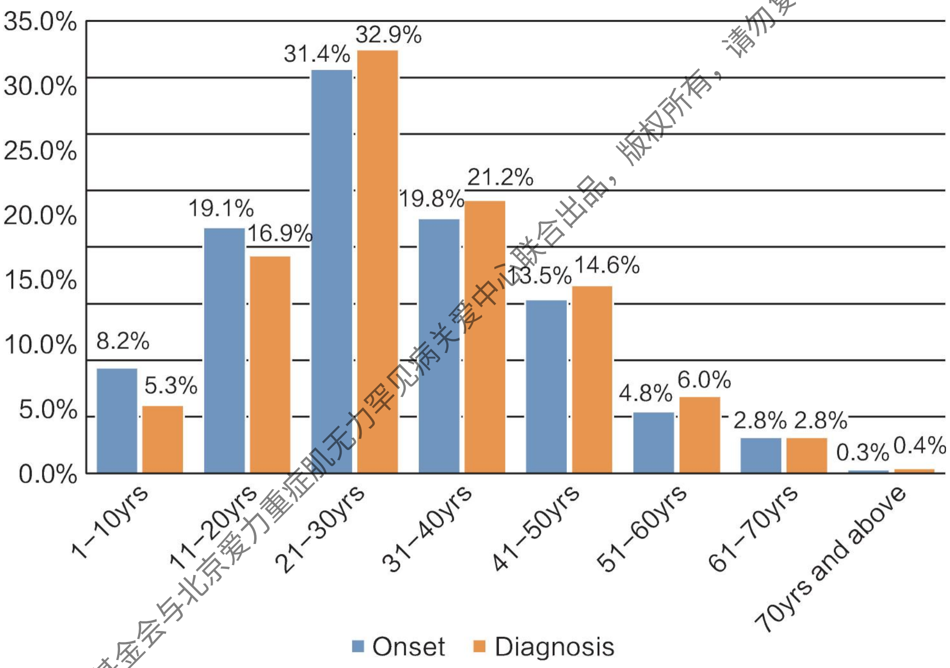
2）Age of onset and age of diagnosis

Among the respondents，the 21-30 year old age group had the largest number of cases，with a total of 320 people，accounting for 31.4%；followed by the 31-40 year old age group，with a total of 202 patients，accounting for 19.8%；the age group over 70 years old had the smallest number，with a total of 3 patients，accounting for 0.3%. The mean age of onset of the patients was 29.4，and the median age of onset was 28.

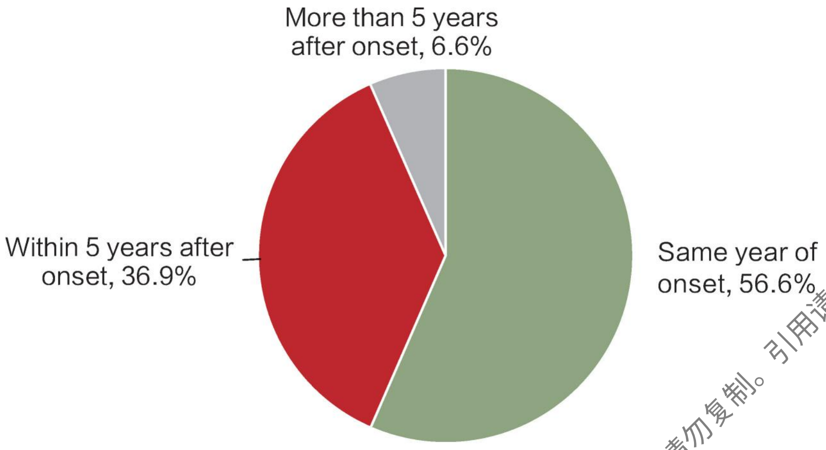




Among the respondents，the21-30 year old age group had the largest number of patients diagnosed with MG，with a total of 336 people，accounting for 32.9%；followed by the 31-40 year old age group，with a total of 216 patients，accounting for 21.2%. The number of patients diagnosed over the age 70 was the least，with a total of 4 people，accounting for 0.4%. The mean age at diagnosis of MG was 31.0 years，and the median age at diagnosis was 29 years.



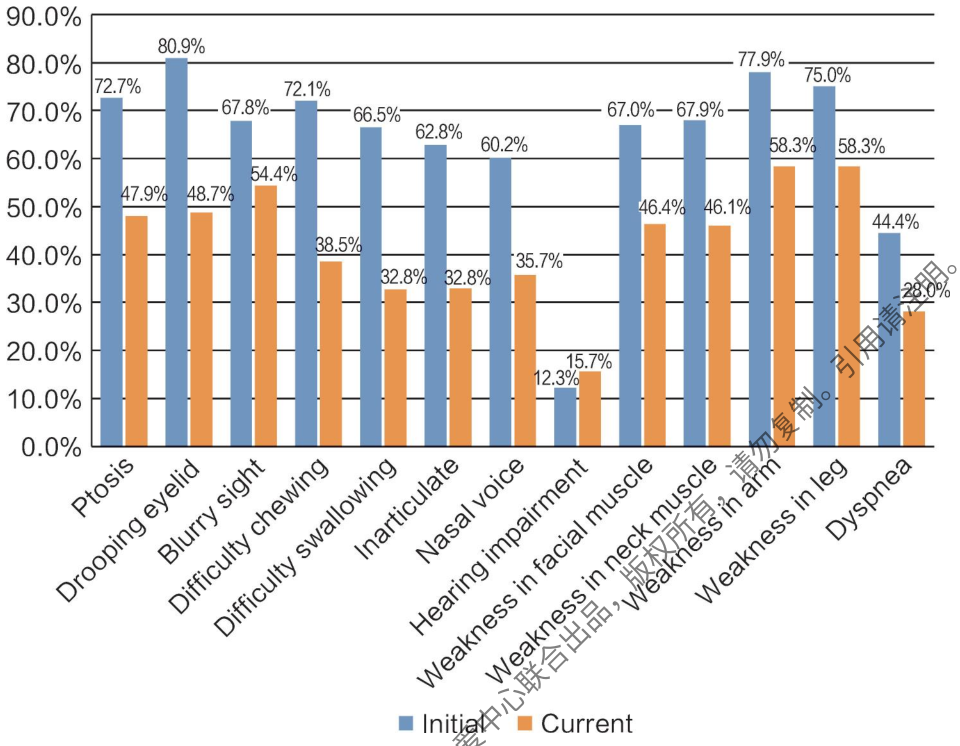
There were577 patients diagnosed with MG in the same year of onset，accounting for 56.6%；376 patients were diagnosed within 1-5 years of onset，accounting for 36.9%；67 patients were diagnosed more than 5 years after onset，accounting for 36.9%.



3）Initial symptoms and current symptoms

The most common initial symptom among therespondents was ptosis，with a total of 825 patients，accounting for 80.9%；followed by arm weakness，with a total of 795 patients，accounting for 77.9%. The least common initial symptom was hearing impairment，with a total of 125 patients，accounting for 12.3%；followed by dyspnea，with a total of 453 patients，accounting for 44.4%.

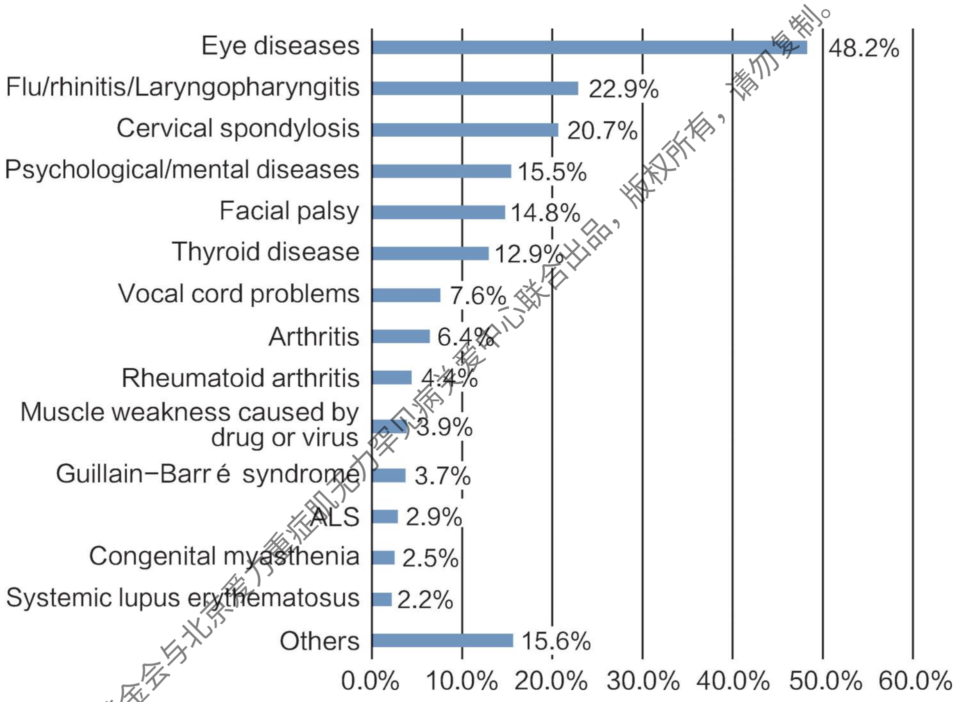
The most common current symptoms among the respondents were arm weakness，leg weakness，both with 595 patients，accounting for 58.3%；the least common current symptom was hearing impairment，with a total of 160 patients，accounting for 15.7%；this was followed by dyspnea，with a total of 286 patients，accounting for 28.0%.



4）Misdiagnosis

Among therespondents，a total of 595 patients had been misdiagnosed，accounting for 58.3%；425 patients had not been misdiagnosed，accounting for 41.7%.

Among the 595 patients who had been misdiagnosed, most patients were misdiagnosed as eye diseases, with a total of 287 patients, accounting for 48.2%; this was followed by flu/rhinitis/larynopharyngitis, with a total of 136 patients, accounting for 22.9%. The patients that were misdiagnosed as systemic lupus erythematosus were the least, with a total of 13 patients, accounting for 2.2%; this was followed by congenital myasthenia, with a total of 15 patients, accounting for 2.5%.

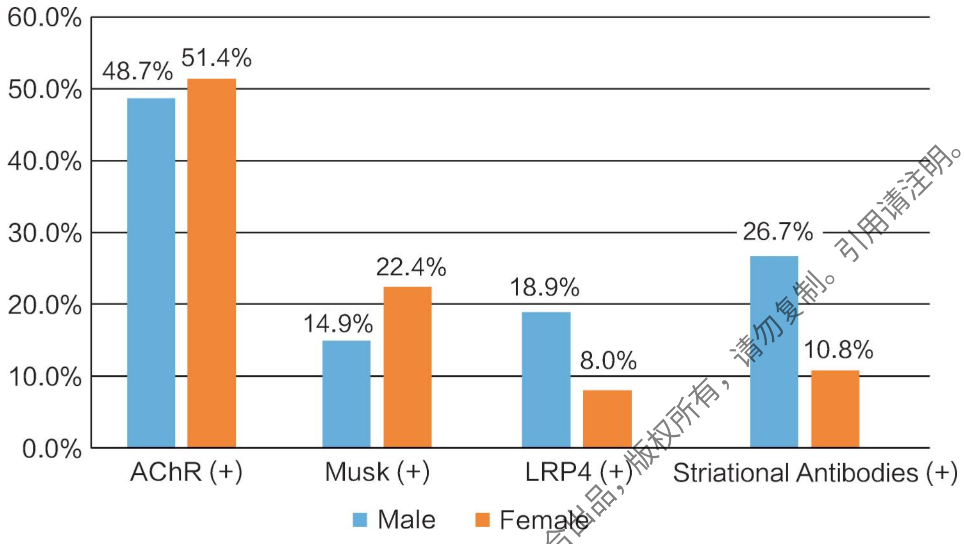


5）Antibody test

Among therespondents, 586 had AchR antibody tests, accounting for 57.5%, of which 297 were positive, accounting for 50.7%; 49 were negative, accounting for 8.4%. Of the 279 patients who had been tested for Musk antibody, 57 were positive, accounting for 20.4%; and 86 were negative, accounting for 30.8%. Of the 143 patients who had been tested for LRP4 antibody，16 were positive，accounting for 11.2%；and 53 were negative，accounting for 37.1%. Of the 138 patients who had been tested for striational antibodies，22 were positive，accounting for 15.9%；and 38 were negative，accounting for 27.5%.

Type	Number	%	Result					
			Positive	%	Negative	%	Not Sure	%
AChR Antibody	586	57.5	297	50.7	49	8.4	240	41.0
Musk Antibody	279	27.4	57	20.4	86	30.8	136	48.8
LRP4 Antibody	143	14.0	16	11.2	53	37.1	74	51.8
Striational Antibodies	138	13.5	22	15.9	38	27.5	78	56.5
None Above	89	8.7	—	—	—	—	—	—
Not Sure	331	32.5	—	—	—	—	—	—

Among the patients who hadtaken AChR antibody test，the proportion of male who tested positive was 48.7%，which was lower than that of female（51.4%）；the proportion of male who tested positive in Musk antibody test was 14.9%，which was lower than that of female（22.4%）；the proportion of male who tested positive in LRP4 antibody test was 18.9%，which was higher than that of female（8.0%）；the proportion of male who tested positive in the striated muscle antibody test was 26.7%, which was higher than that of female (10.8%).



6) Diagnostic testing

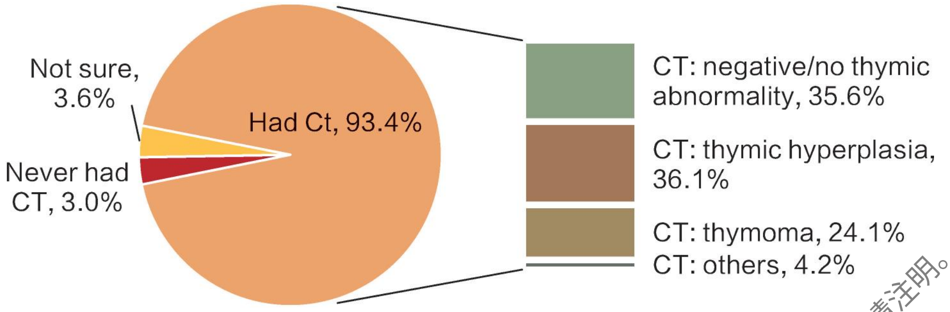
Among the respondents, 839 had EMG tests, accounting for 82.3%, of which 457 were positive, accounting for 54.5%; and 65 were negative, accounting for 7.7%. Of the 754 patients who had neostigmine test, 524 were positive, accounting for 69.5%; and 15 were negative, accounting for 2.0%. Of the 323 patients who had test for fatigue, 215 were positive, accounting for 66.6%; and 15 were negative, accounting for 4.6%.

Type	Number	%	Result					
			Positive	%	Negative	%	Not Sure	%
EMG	839	82.3	457	54.5	65	7.7	317	37.8

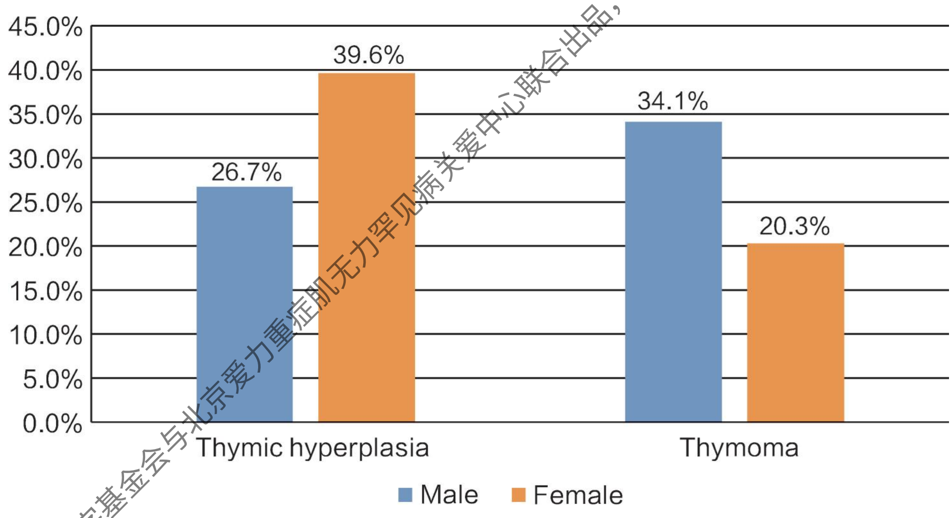
Type	Number	%	Result					
			Positive	%	Negative	%	Not Sure	%
Neostigmine	754	73.9	524	69.5	15	2.0	215	28.5
Fatigue	323	31.7	215	66.6	15	4.6	93	28.8
Sleep/Rest	51	5.0	19	37.3	2	3.9	30	58.8
Tensilon	26	2.6	18	69.2	1	3.8	7	26.9
Ice Pack	18	1.8	12	66.7	5	27.8	1	5.6
None of the Above	14	1.4	—	—	—	—	—	—
Not Sure	59	5.8	—	—	—	—	—	—

7）Chest CT examination

Among therespondents，952 patients had chest CT examination，accounting for 93.4%；and 31 patients had not taken chest CT examination，accounting for 3.0%. Among the 952 patients who had taken chest CT examination，the number of patients with thymic hyperplasia was the largest，with 344 patients，accounting for 36.1%；followed by patients without abnormality，with 339 patients，accounting for 35.6%；and 229 patients had the examination results as thymoma，accounting for 24.1%.



The proportion of male patients with thymic hyperplasia was 26.7%，which was lower than that offemale（39.6%）；and the proportion of male patients with thymoma was 34.1%，which was higher than that of female（20.3%）.



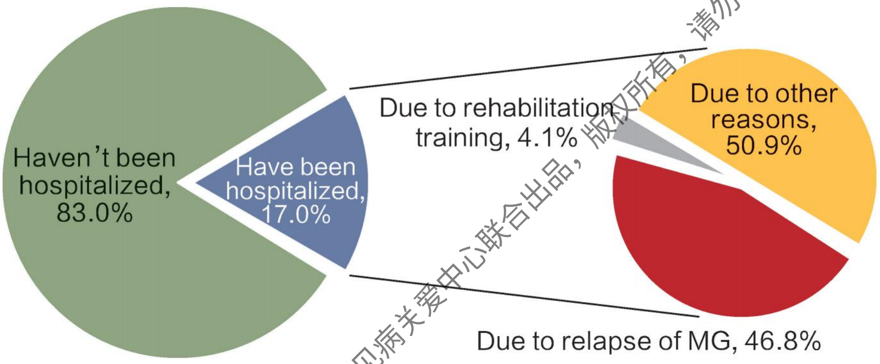
6. Hospitalization and medical treatment experience

1）Hospitalization

In the past six months，173 patients had been hospitalized，accounting for

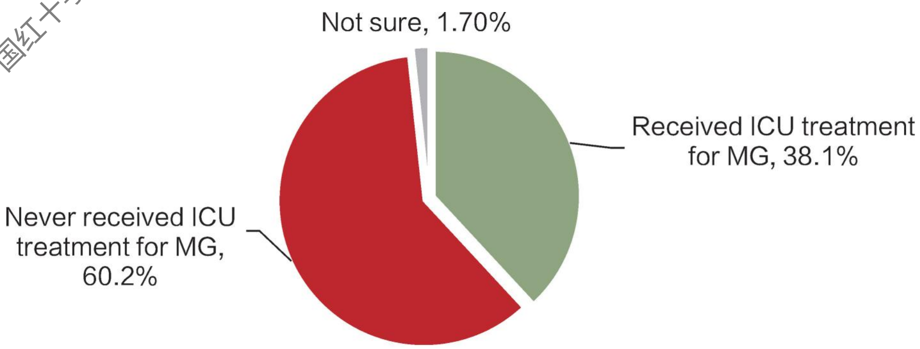
17.0%；and 847 patients had not been hospitalized，accounting for 83.0%. Among the 173 patients who had been hospitalized，81 were hospitalized due to the recurrence of MG，accounting for 46.8%；and 7 patients were hospitalized due to rehabilitation training，accounting for 4.1%.

Of the 88 patients who had been hospitalized for other reasons，6 patients were hospitalized for follow-ups（6.8%）and 6 patients were hospitalized for routine care（6.8%）. There were 4 patients were hospitalized for flu（4.5%）and 3 patients were hospitalized for complications（3.4%）.

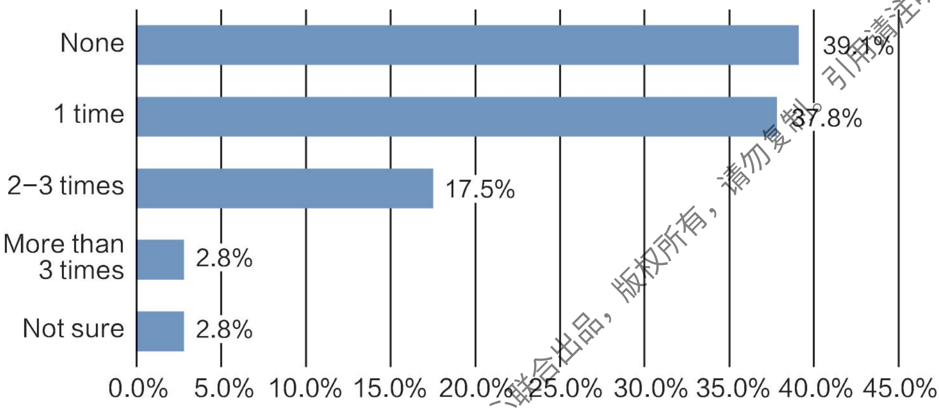


2）ICU treatment and crisis

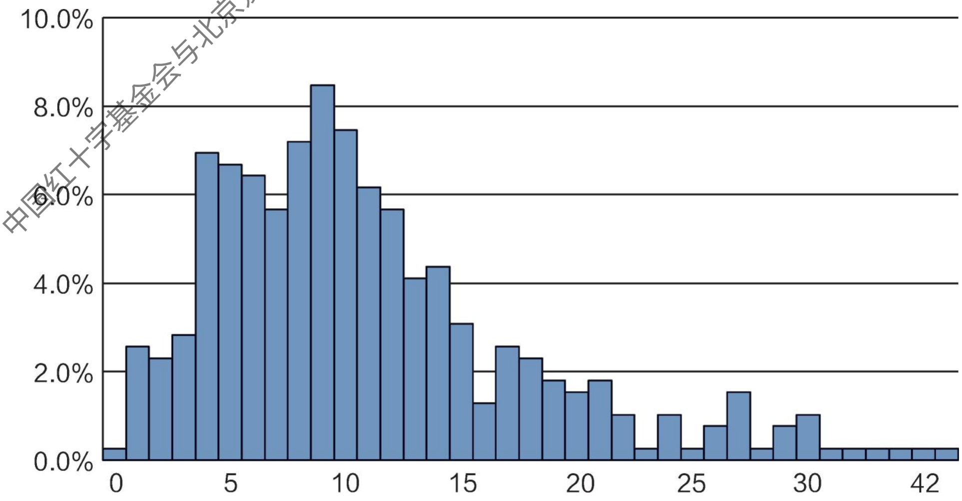
Among the respondents，389 had received ICU treatment for MG，accounting for 38.1%；and 614 had never received ICU treatment for MG，accounting for 60.2%.



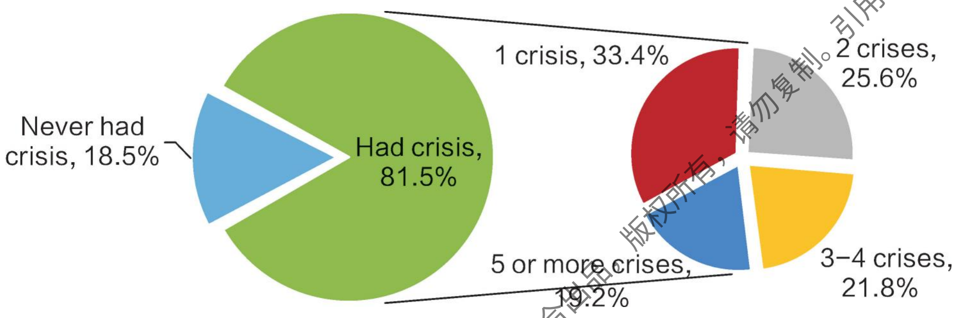
Among the 389 patients who had received ICU treatment for MG，152 had no ICU treatment in the past five years，accounting for 39.1%；147 had one ICU treatment，accounting for 37.8%；68 had 2-3 ICU treatments，accounting for 17.5%；and 11 people had more than 3 ICU treatments，accounting for 2.8%.



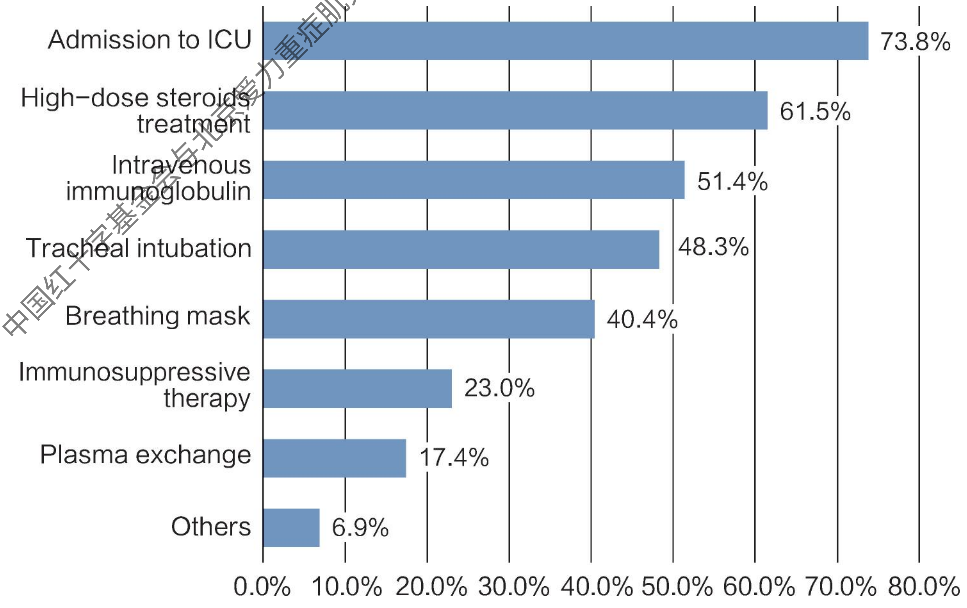
The graph below shows the distribution of the duration of illness for 389 patients who received ICU treatment within five years. The highest percentage of patients，48.8%，had a disease duration between 4 and 10 years. The proportion of patients with a disease duration of 15 years or more was lower.



Among the 389respondents who had been treated in the ICU，317 patients had experienced crisis，accounting for 81.5%；72 patients had never experienced crisis，accounting for 18.5%. Of those who experienced crisis，most had one crisis，with a total of 106 people，accounting for 33.4%，followed by those who had 2 crises，with a total of 81 people，accounting for 25.6%；and 61 people had 5 or more crises，accounting for 19.2%.

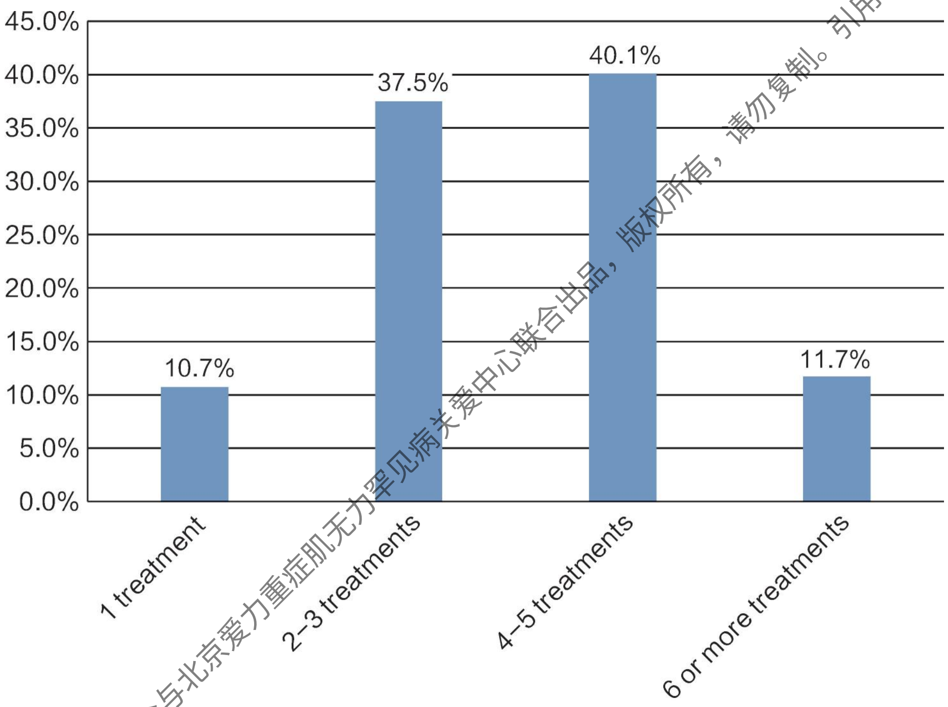


Among the 317 patients who had a crisis，the most common rescue measure was admission to the ICU，which was reported by 234 patients，



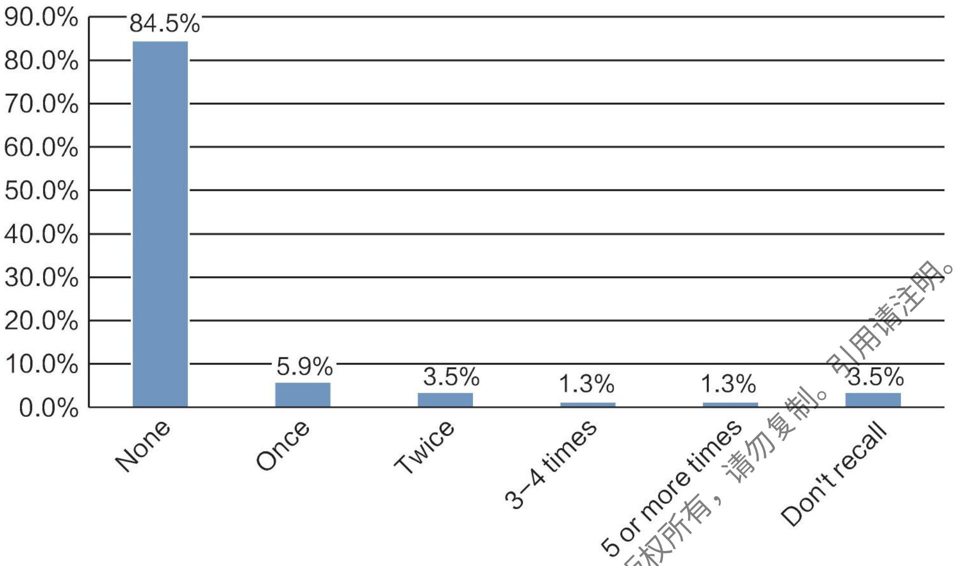
accounting for 73.8%; followed by high-dose steroid treatment, reported by 153 patients, accounting for 61.5%; and the least common rescue measure was plasma exchange, reported by 55 patients, accounting for 17.4%.

After crisis, 10.7% of patients received one treatment, 37.5% received 2-3 treatments, 40.1% received 4-5 treatments, and 11.7% received 6or more treatments.



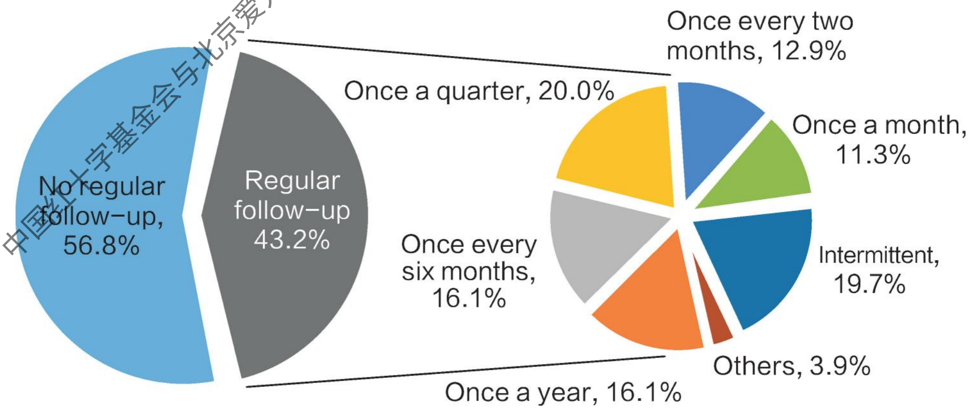
3) Emergency treatment

In the past six months, 862 patients had not been to the emergency department, accounting for 84.5%; 60 patients had been to emergency department once, accounting for 5.9%; and 13 patients had been to emergency department at least 5 times, accounting for 1.3%.



4）Follow-up

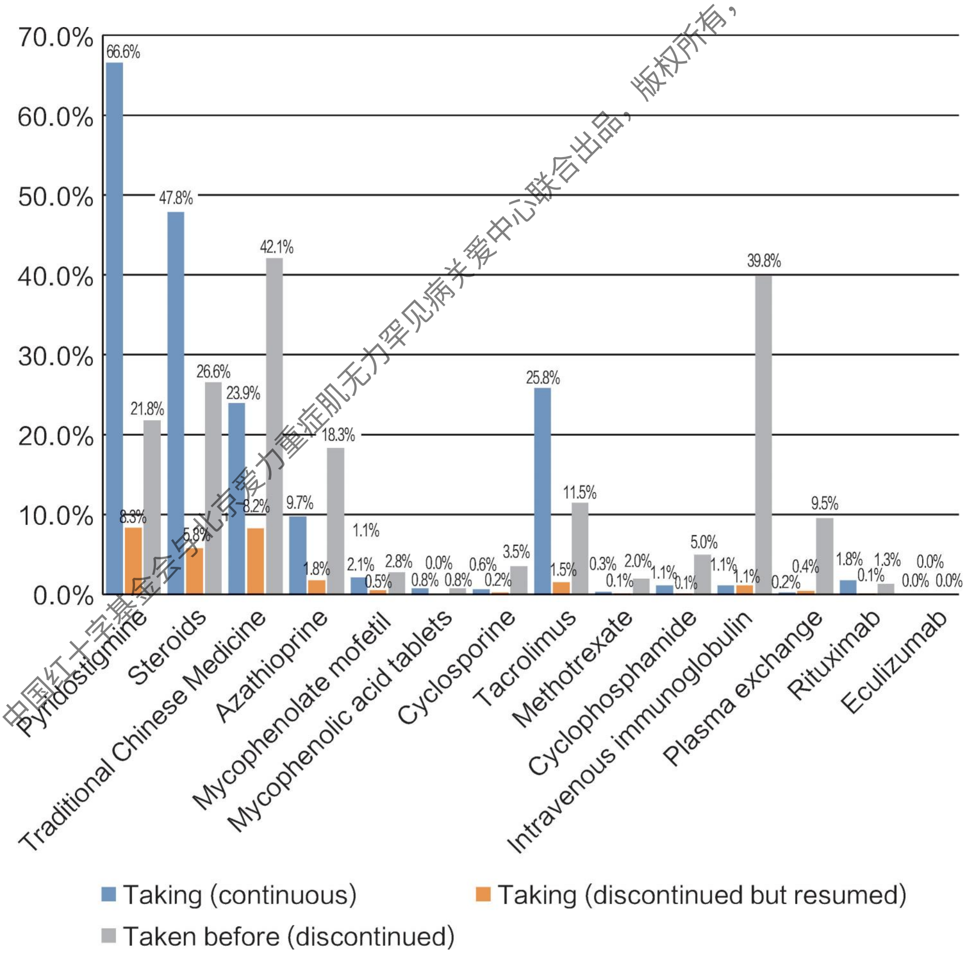
Among the respondents，441 visited the hospital for follow-up on a regular basis after diagnosis，accounting for 43.2%. Among them，the most common frequency of follow-up was once a quarter，reported by 88 patients，accounting for 20.0%；and 87 patients had intermittent follow-up，accounting for 19.7%.



7. Medication

1）Drug treatment and use

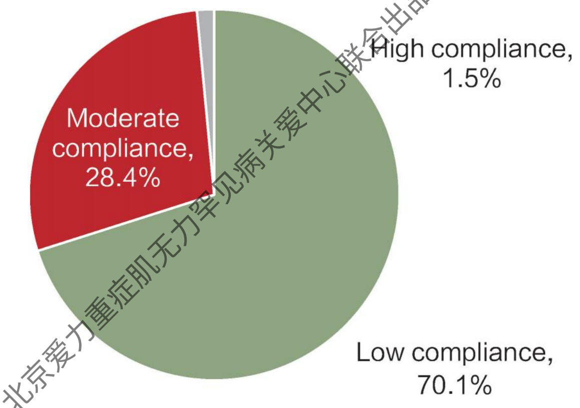
Among the respondents，679 patients were using pyridostigmine bromide，accounting for 66.6%；this was followed by patients using steroids，with a total of 488，accounting for 47.8%. The number of patients who were using methotrexate was the least，with a total of 3 people，accounting for 0.3%.



2）Medication Adherence-MMAS-8

In this survey，the Morisky Medication Adherence Scale（MMAS-8）was used to evaluate the medication adherence of patients taking medication（see the appendix for the scale）. The total score of the scale ranges from 0-8，with a higher score indicating better medication adherence.

The results showed that among the 946 patients，MMAS-8 scores ranged from 0.25 to 8.00，with a mean of 4.70 and a median of 4.75. Only 1.5% of patients had high medication adherence（8 points）；28.4% of patients had moderate medication adherence（6-7 points）；and 70.1% of patients had low medication adherence（<6 points）.



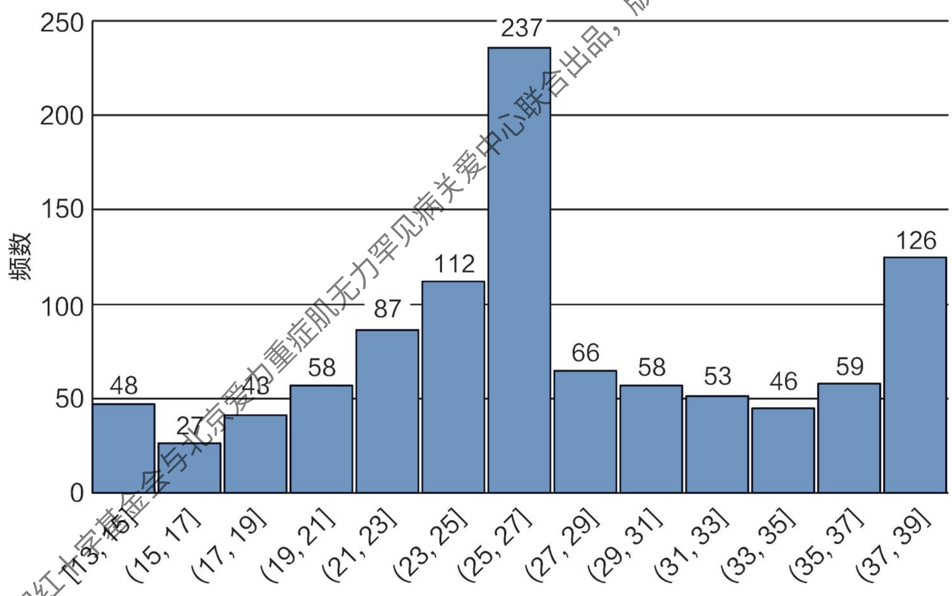
Mean	Median	Minimun	Maximun
4.70	4.75	0.25	8.00

3）Medication Adherence-SEAMS

This survey used the Self-efficacy for Appropriate Medication Use

(SEAMS) to evaluate patients' medication adherence (see the appendix for the scale). The scale has good reliability and validity in measuring self-efficacy of medication adherence and is suitable to be used for patients with various types of chronic diseases. The scale consists of two dimensions (medication taking in uncertain situations and difficult situations) with 13 items and a total score ranging from 13 to 39, with higher score indicating higher medication adherence.

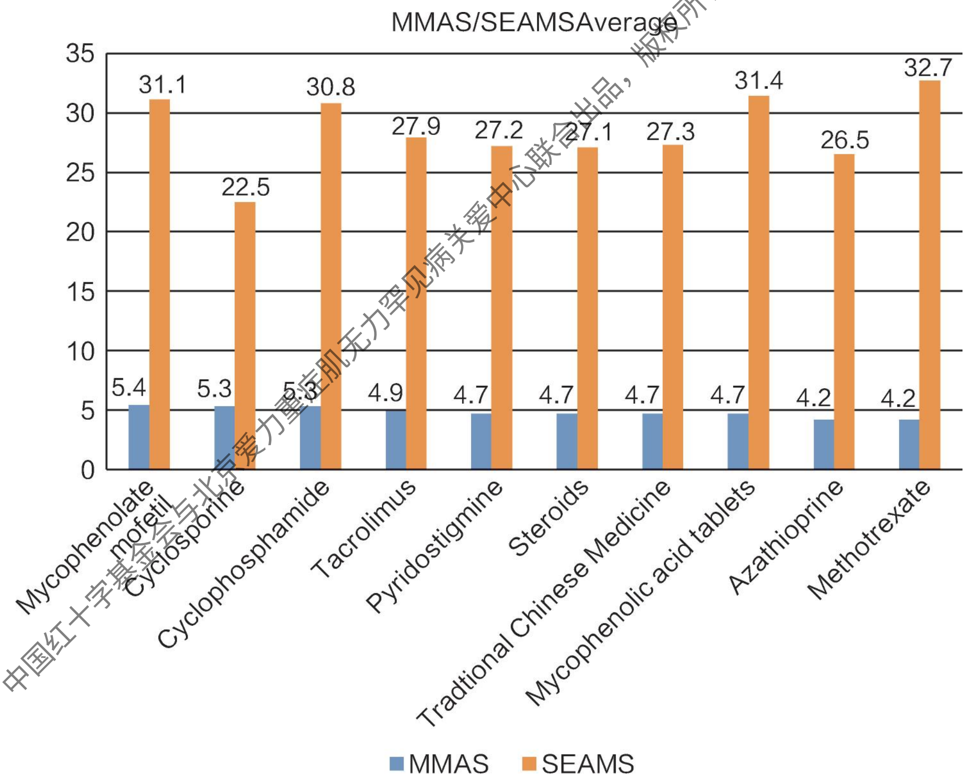
The results showed that among the 1020 respondents, SEAMS scores ranged from 13 to 39, with a mean of 27.5 and a median of 26. The higher the score, the higher the patient's confidence in self-medication management.



Mean	Median	Minimun	Maximun
27.5	26	13	39

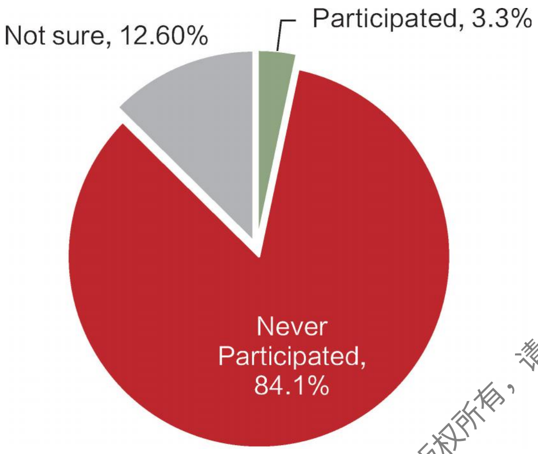
4）Medication adherence of patients using various types of medication

Among all the patients on medication，patients on mycophenolate mofetil had the highest MMAS score，followed by patients on cyclosporine and cyclophosphamide，while patients on azathioprine and methotrexate had the lowest MMAS score. In terms of the SEAMS mean score，the patients on methotrexate and mycophenolic acid tablets had the highest mean scores，while the patients on cyclosporine and azathioprine had the lowest mean scores.

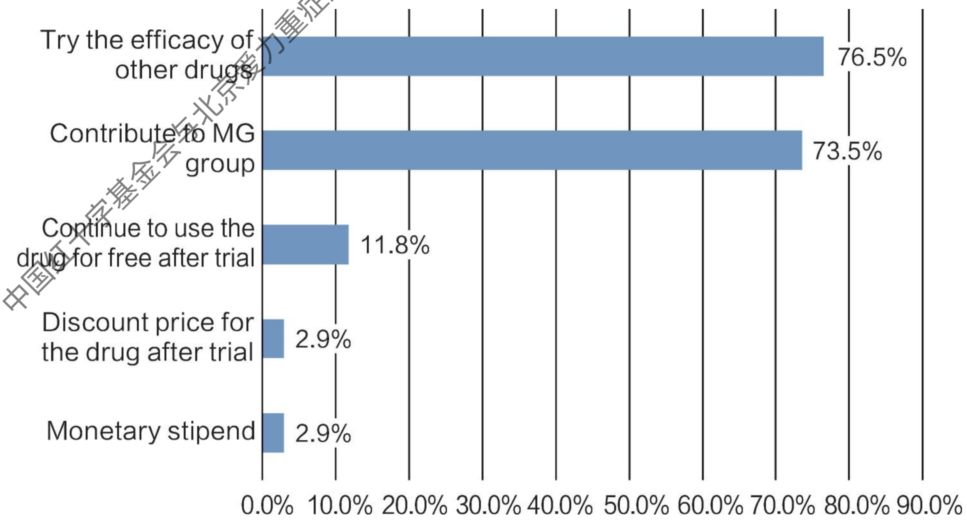


5）Participation in drug clinical trials

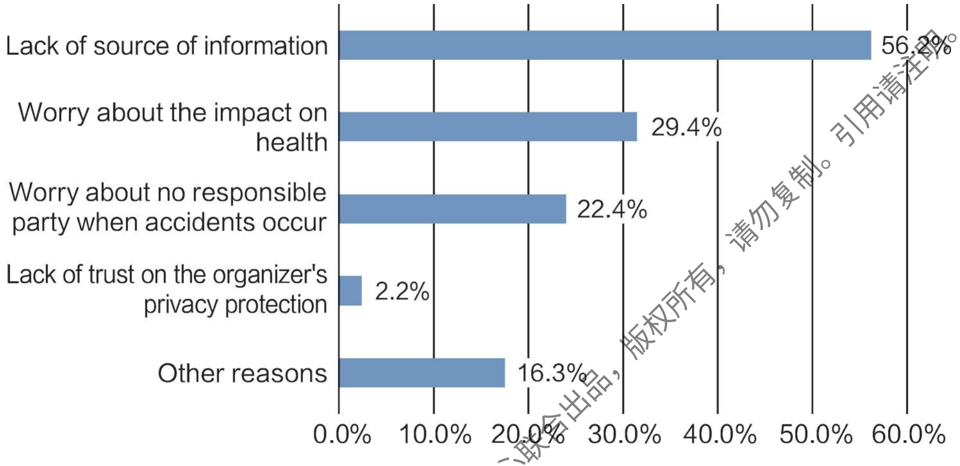
Among the respondents，34 had participated in clinical trials of MG drugs， accounting for 3.3%; while 858 patients had never participated in clinical trials of MG drugs, accounting for 84.1%.



Among the 34 patients who had participated in clinical trials of MG drugs, 76.5% believed that the benefit of participating in the clinical trial was that they could try the efficacy of the experimental drug; and 73.5% of the patients believed that the benefit of participating in the clinical trial was to contribute to the MG community.



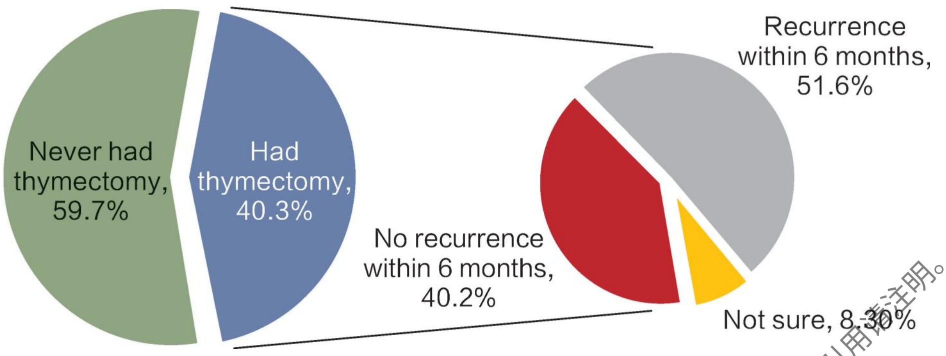
Among the 858 patients who had never participated in clinical trials, the most common reason for not participating was lack of information about the clinical trials，which was reported by 482 patients，accounting for 56.2%；and the second reason was concerns about the impact of clinical trials on health，reported by 252 people，accounting for 29.4%.



8. Surgery and other treatments

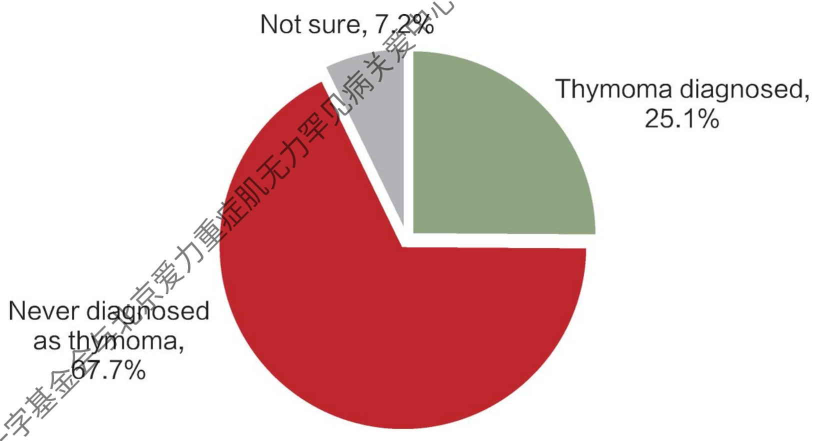
1）Thymectomy

Among the respondents，411 patients had undergone thymectomy，accounting for 40.3%；while 609 patients had not undergone thymectomy，accounting for 59. 7%. Among the 411 patients who had undergone thymectomy，212 experienced disease recurrence or exacerbation within six months after surgery，accounting for 51.6%；while 165 patients did not experience disease recurrence or exacerbation within six months，accounting for 40.2%.

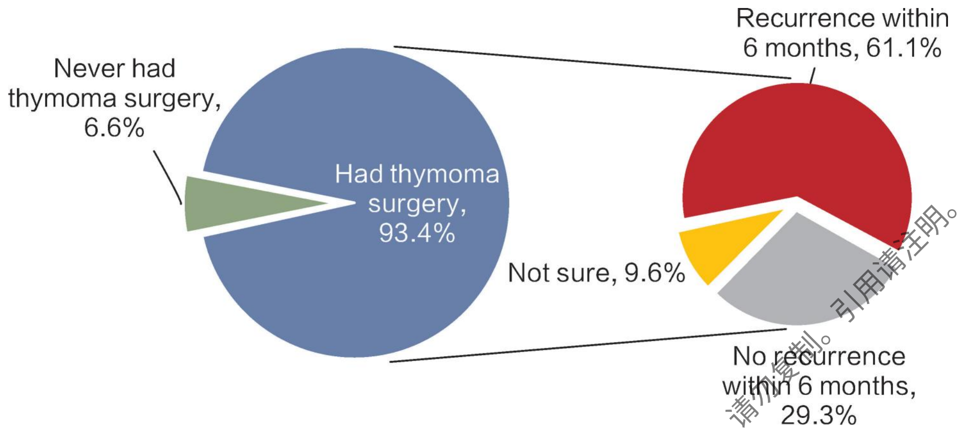


2）Thymoma surgery

Among therespondents，256 had been diagnosed with thymoma，accounting for 25.1%；while 691 had never been diagnosed with thymoma，accounting for 67.7%.

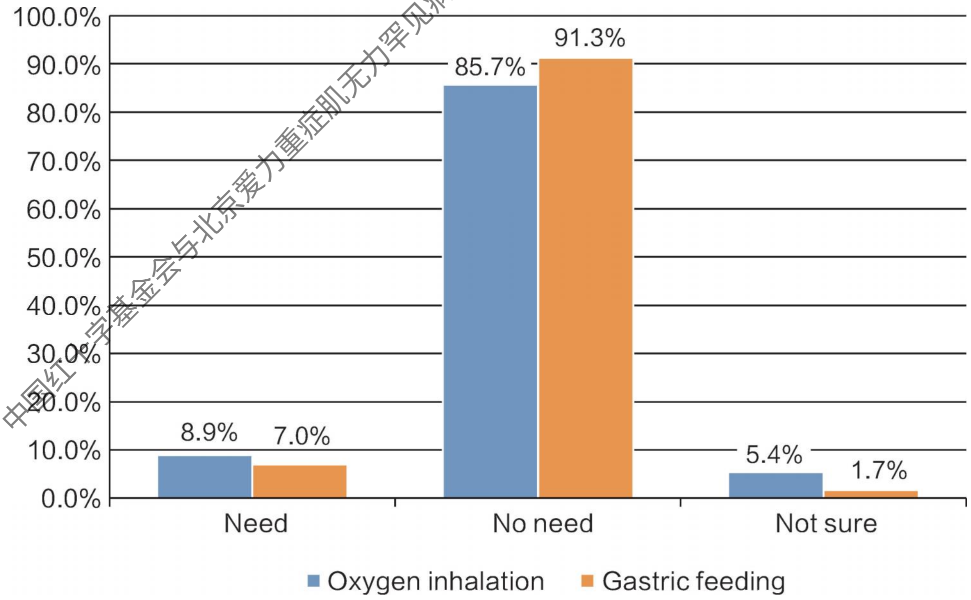


Among the 256respondents diagnosed with thymoma，239 patients had undergone thymoma surgery，accounting for 93.4%；while 17 patients had not undergone thymoma surgery，accounting for 6.6%. Among the 239 patients who had undergone thymoma surgery，146 experienced disease recurrence or exacerbation within six months after surgery，accounting for 61.1%；while 70 patients did not experience disease recurrence or exacerbation within six months，accounting for 29.3%.



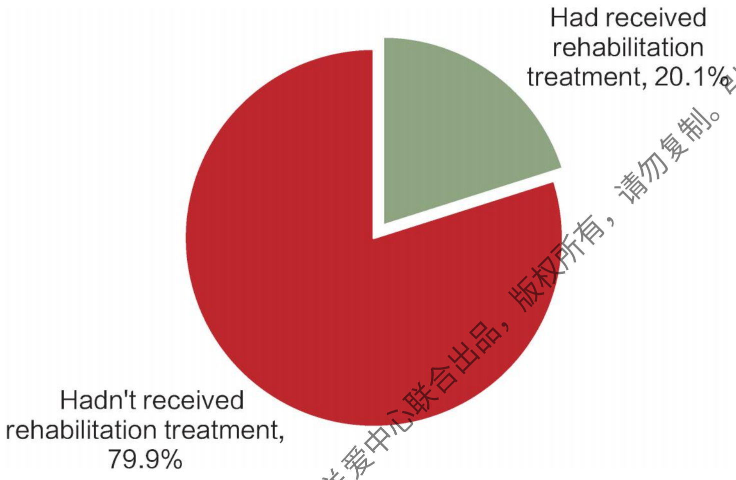
3）Oxygen inhalation/gastric feeding

Among the respondents，91 patients required oxygen therapy，accounting for 8.9%；and 71 patients required gastric feeding，accounting for 7.0%.

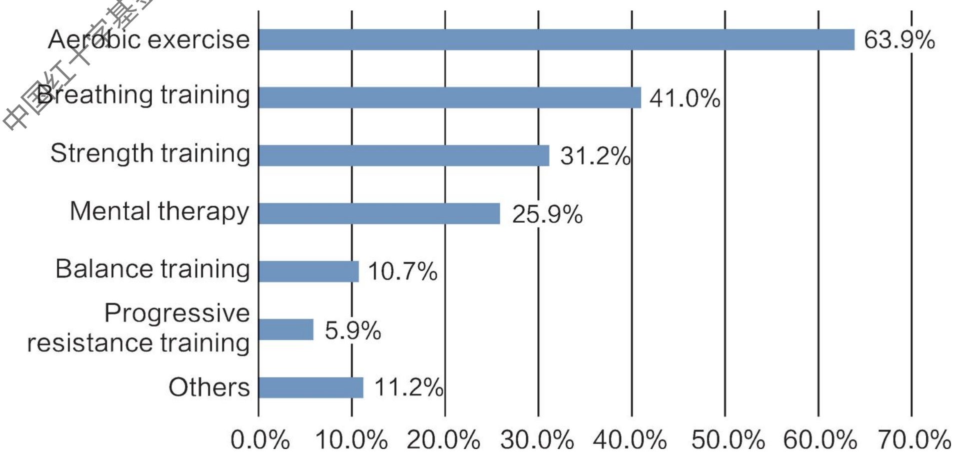


4）Rehabilitation

Among the respondents，205 had received rehabilitation treatment，accounting for 20.1%；while 815 patients had not received rehabilitation treatment，accounting for 79.9%.

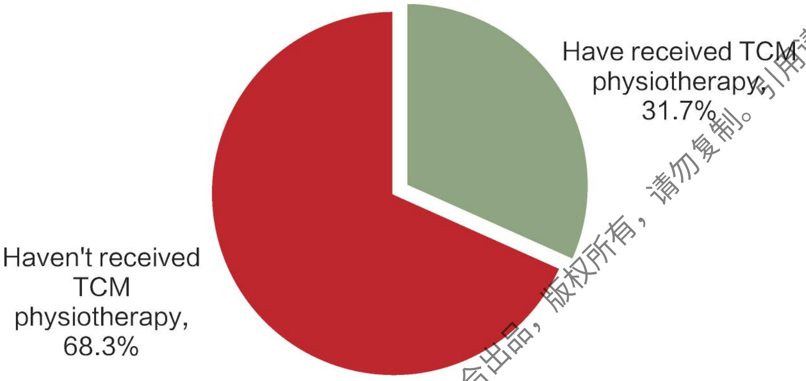


Among the 205 people who received rehabilitation treatment，the most common rehabilitation treatment was aerobic exercise，received by 131 patients，accounting for 63.9%；followed by breathing training，received by 84 patients，accounting for 41.0%；and the least common rehabilitation treatment was progressive resistance training，received by 12 patients，accounting for 5.9%.

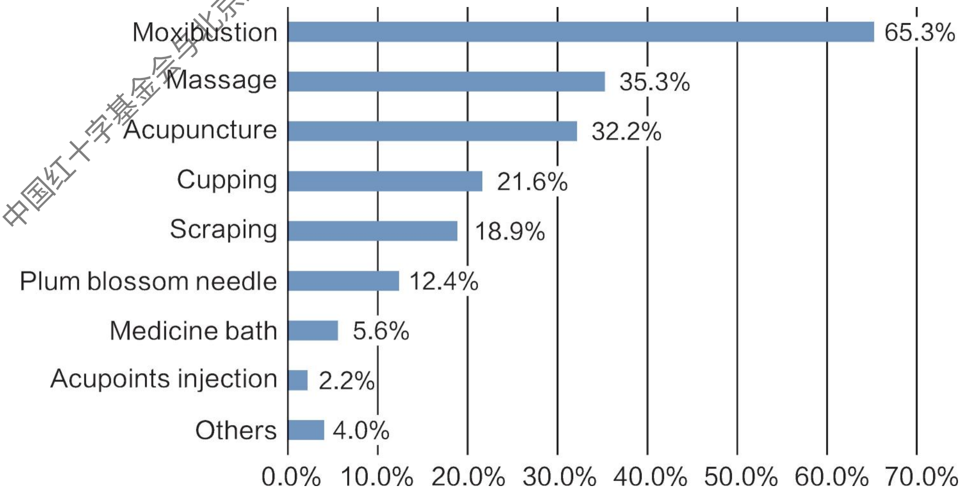


5）Traditional Chinese Medicine Physiotherapy

Among the respondents，323 had received TCM physiotherapy，accounting for 31.7%；while 697 had not received TCM physiotherapy，accounting for 68.3%.

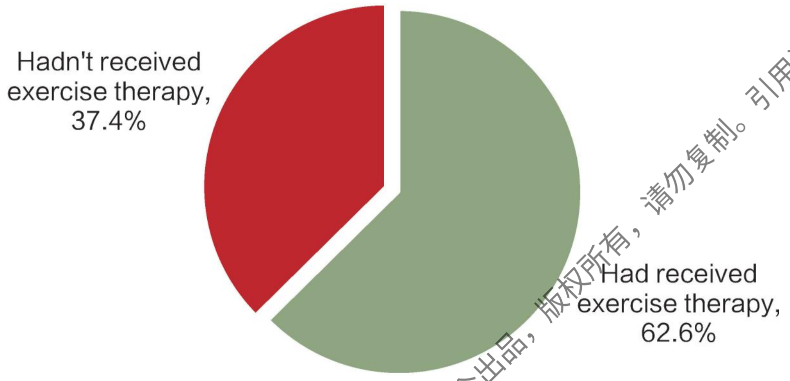


Among the 323 patients who had received TCM physiotherapy，the most common TCM physiotherapy was moxibustion，received by 211 patients，accounting for 65.3%；followed by massage，received by 114 patients，accounting for 35.3%；and the least common TCM physiotherapy was acupoints injection，received by 7 patients，accounting for 2.2%.

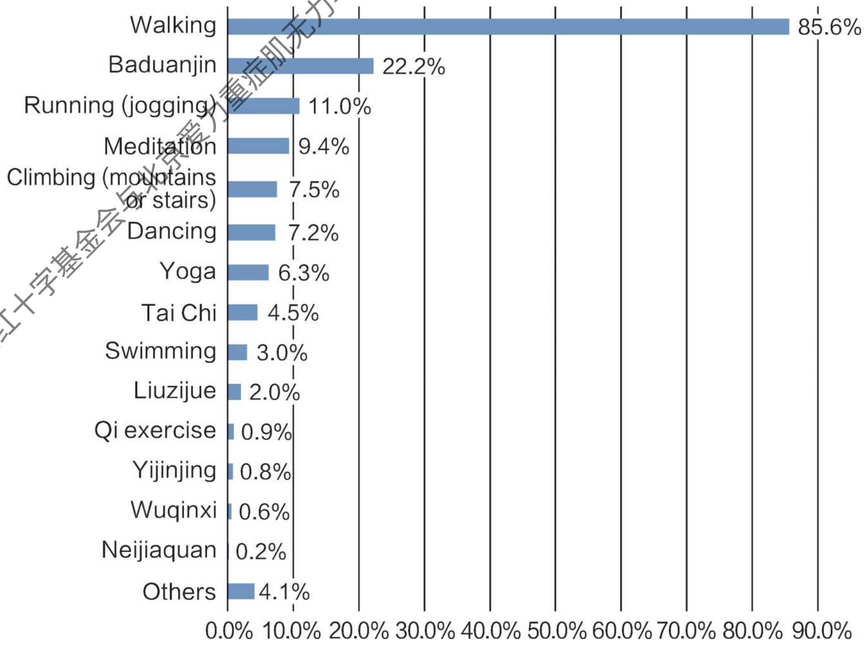


6）Exercise therapy

Among the respondents，639 had received exercise therapy，accounting for 62.6%；while 381 patients had not received exercise therapy，accounting for 37.4%.



Among the 639 patients who received exercise therapy，the most common exercise therapy was walking，adopted by 547 patients，accounting for 85.6%；followed by Baduanjin，adopted by 142 patients，accounting for 22.2%；and



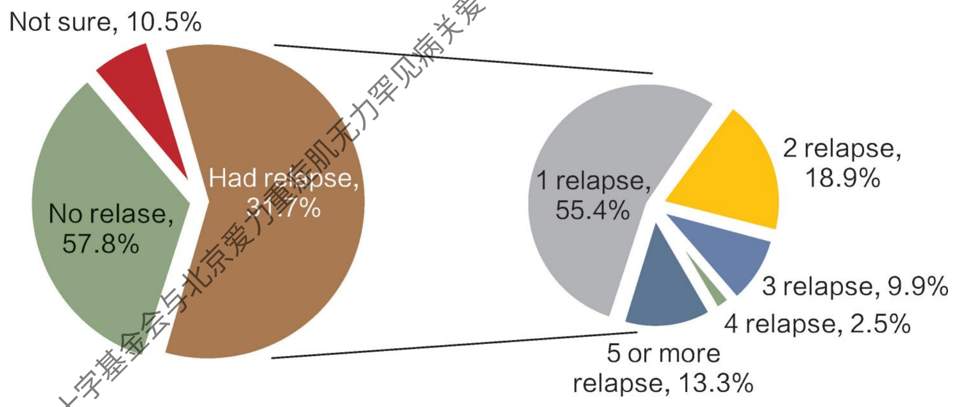
the least common exercise therapy was Neijiaquan，adopted by 1 patient，accounting for 0.2%.

9. Relapse and change in disease condition

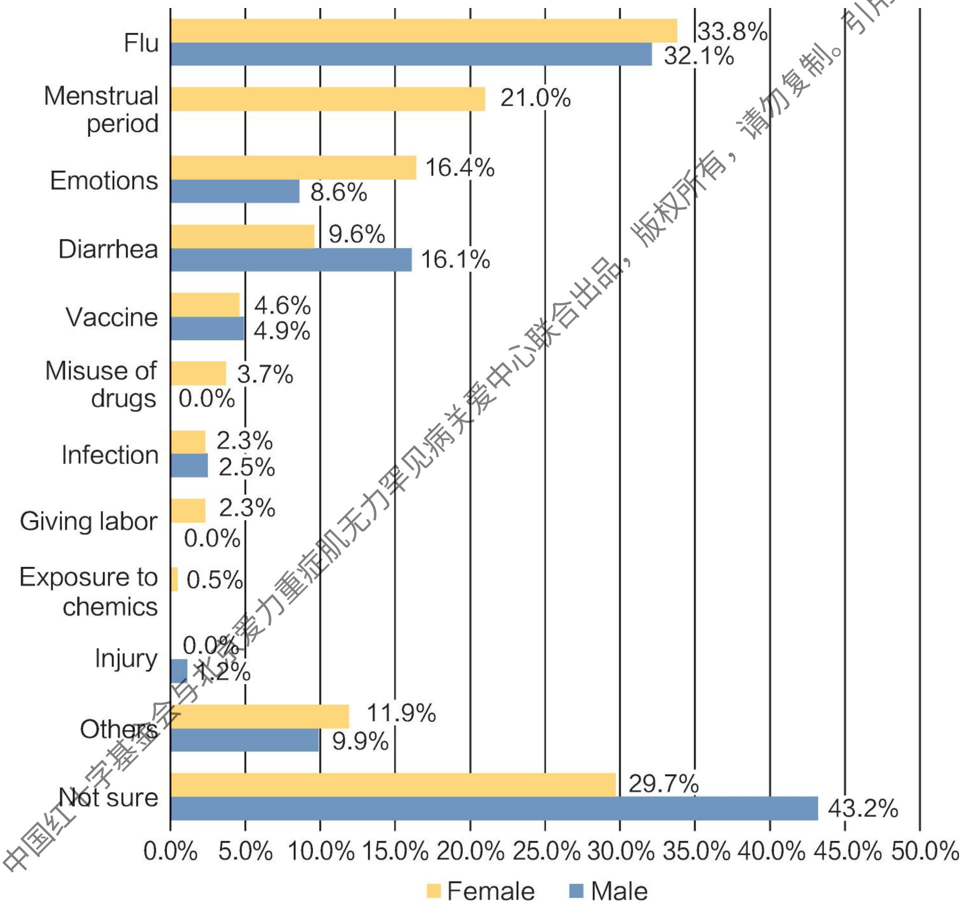
1）Relapse and reasons for relapse

In the past six months，300 patients had relapse，accounting for 29.4%；while 613 patients had no relapse，accounting for 60.1%.

Among the 300 patients with disease relapse in the past six months，most had one relapse，with a total of 179 patients，accounting for 59.7%；while the patients with 4 relapses were the least，with a total of 5 patients，accounting for 1.7%.

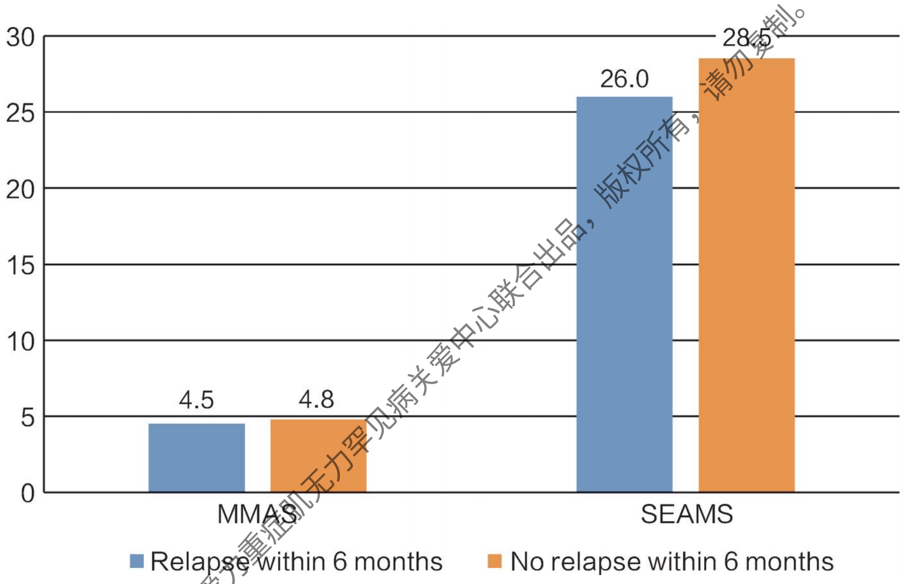


Of the 300 patients who had relapsed within the past six months，219 were females and 81 were males. Among female patients，the most common cause of recurrence was flu，reported by 74 patients，accounting for 33.8%；followed by menstruation，reported by 46 patients，accounting for 21.0%；and the least common cause was exposure to chemical substances，reported by 1 patient，accounting for 0.5%. No female patients had relapse caused by trauma. Among male patients，the most common cause of recurrence was flu，reported by 26 patients，accounting for 32.1%；followed by diarrhea，reported by 13 patients，accounting for 16.1%；and the least common cause was exposure to chemical substances，accounting for 0%. Relapse due to emotional reasons was more common among female patients（16.4%）than male patients（8.6%）.



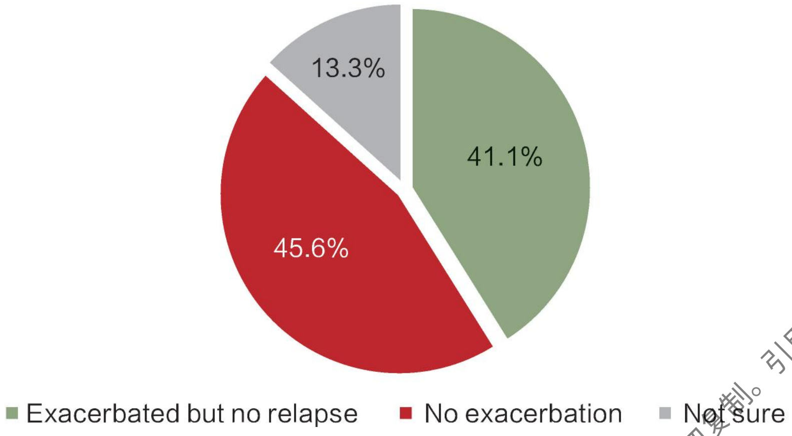
2）Relapse and medication adherence

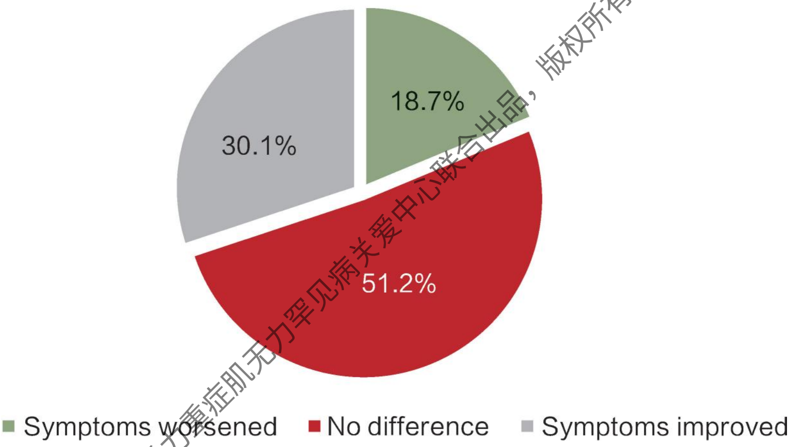
When comparing the medication adherence of relapsed patients with non-relapsed patients，the results showed that patients with relapse within the past six months had a mean MMAS score of 4.5 and a mean SEAMS score of 26.0；while patients without recurrence within six months had a mean MMAS score of 4.8 points，and a mean SEAMS score of 28.5 points. The mean scores MMAS and SEAMS of relapsed patients were lower than those of non-relapsed patients，and the difference was statistically significant. This result suggests a preliminary correlation between medication adherence and disease relapse，but its actual association needs to be verified in further analysis.



3）Changes in disease condition

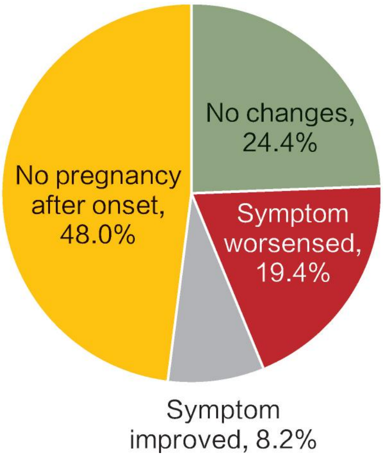
In the past six months，419 patients experienced exacerbation but not relapse，accounting for 41.1%；and 465 patients did not experience exacerbation，accounting for 45.6%. There were 191 patients who believed that their current symptoms had worsened compared to six months ago，accounting for 18.7%；522 patients believed that their symptoms had not changed，accounting for 51.2%；and 307 patients believed that their symptoms had improved，accounting for 30.1%.





4) Female pregnancy and childbirth and illness

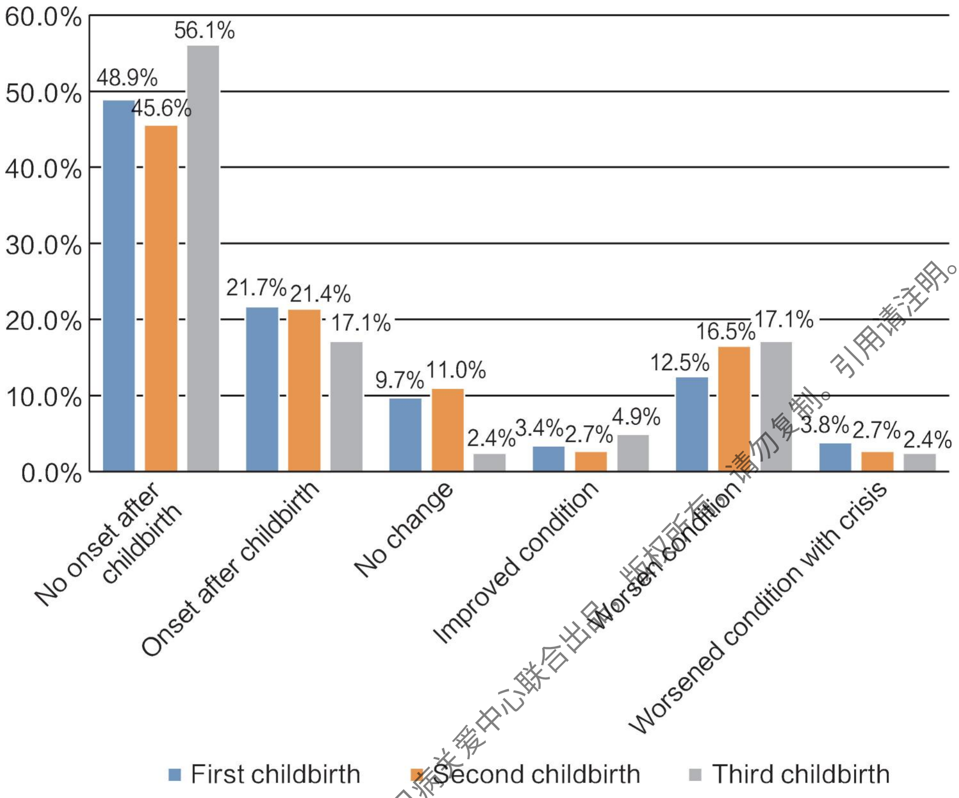
Among the respondents, 537 female patients had been pregnant. Among them, 131 patients had no change in myasthenia gravis condition during pregnancy, accounting for 24.4%; 104 patients had worsened conditions during pregnancy, accounting for 19.4%; 44 patients had symptoms improved during pregnancy, accounting for 8.2%.



Among the respondents，a total of 497 female patients had experienced childbirth. Among them，after the first childbirth，62 patients had worsened condition but without crisis，accounting for 12.5%；19 patients had worsened condition and crisis，accounting for 3.8%；48 patients had no change in their condition，accounting for 9.7% %；and 17 patients had improved condition，accounting for 3.4%.

Among the 182 female patients who experienced second childbirth，after the second childbirth，30 patients had worsened condition but without crisis，accounting for 16.5%；5 patients had worsened conditions and crisis，accounting for 2.7%；20 patients had no change in their condition，accounting for 11.0%；and 5 patients had improved condition，accounting for 2.7%.

Among the 41 female patients who experienced the third childbirth，after the third childbirth，7 patients' conditions worsened but without crisis，accounting for 17.1%；1 patient had worsened condition and crisis，accounting for 2.4%；1 patient had no change in the condition，accounting for 2.4%；and 2 patients had improved condition，accounting for 4.9%.

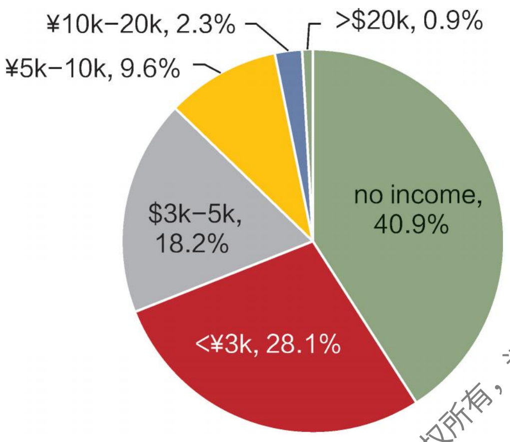


10. Patient financial status

1）Patient’s personal monthly income

Among all the respondents，417 had no income at present，accounting for 40.9%；287 reported monthly income of less than ￥3，000，accounting for 28.1%；186 reported monthly income of ￥3，000-5，000，accounting for 18.2%；98 reported monthly income ￥5，000-10，000；23 reported monthly income of ￥10，000-20，000，accounting for 2.3%；and 9 reported monthly income of more than ￥20，000，accounting for 0.9%.

Among the 603 patients with income，the personal monthly income ranged from ￥119-100，000， the average monthly income was ￥4，426， and the median was ￥3，000.

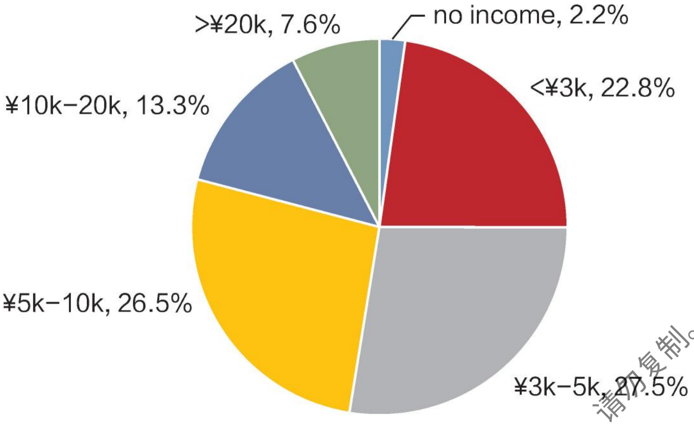


Mean	Median	Minimun	Maximun
￥4，426	￥3，000	￥119	￥100，000

2）Patients’ household monthly income

Among all the respondents， 22 had no household monthly income， accounting for 2.2%； 233 reported monthly household income of less than ￥3，000， accounting for 22.8%； 281 reported monthly income of ￥3，000-5，000， accounting for 27.5%； 270 reported monthly income of ￥50，000-10，000， accounting for 26.5%； 136 reported monthly income of ￥10，000-20，000， accounting for 13.3%； and 78 reported monthly income of more than ￥20，000， accounting for 7.6%.

Among the 998 patients with monthly household income， the total monthly household income ranged from ￥110 to 300，000， the average monthly household income was ￥10,646, and the median was ￥5,000.①



Mean	Median	Minimun	Maximun
￥10,646	￥5,000	￥110	￥300,000

After deducting household daily expenses, the disposable household monthly income ranged from ￥0-290,000, with a mean of ￥6,503.8 and a median of ￥2,000.

Mean	Median	Minimun	Maximun
￥6503.8	￥2,000	￥0	￥290,000

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① Note: If the patients with the lowest and highest 5% of the total monthly household income were excluded, the total household monthly income of the remaining 682 patients ranged from ￥3,008-27,000, the average monthly household income was ￥8,532.9, and the median was ￥7000.

11. Medical expenses

1）Economic Burden of Oral Drugs-Monthly Expenses

- Among the 764 patients taking pyridostigmine bromide，monthly expenditures for the drug ranged from ￥0-5200，with an average monthly expenditure of ￥207.4 and a median of ￥150.

- Among the 547 patients using steroids，the monthly expenditure on the drug ranged from ￥0-600，with an average monthly expenditure of ￥51.6 and a median of ￥15.

- Among the 328 patients using traditional Chinese medicine/proprietary Chinese medicine，the monthly expenditure on the drug ranged from ￥50-30，000，with an average monthly expenditure of ￥1，911 and a median of ￥1，500.

- Among the 278 patients using tacrolimus，the monthly expenditure on the drug ranged from ￥200-6000，with an average monthly expenditure of ￥1366.3 and a median of ￥1200.

- Among the 117 patients using azathioprine，the monthly expenditure on the drug ranged from ￥0-10，000，with an average monthly expenditure of ￥259.3 and a median of ￥108.

- Among the 26 patients using mycophenolate mofetil，the monthly expenditure on the drug ranged from ￥350-2500，with an average monthly expenditure of ￥1024.9 and a median of ￥1000.

- Among the 11 patients on cyclophosphamide，the monthly expenditure on the drug ranged from ￥0-3，000，with an average monthly expenditure of ￥512.3 and a median of ￥247.5.

- Among the 8 patients using cyclosporine，the monthly expenditure on the drug ranged from ￥100-1600 ￥，with an average monthly expenditure of ￥873.1 and a median of ￥750.

- Among the 8 patients using mycophenolic acid tablets，the monthly expenditure on the drug ranged from ￥330-1600，with an average monthly expenditure of ￥948 and a median of ￥890.

- Among the 4 patients on methotrexate，monthly expenditure on the drug ranged from ￥ 30-90，with an average monthly expenditure of ￥52.5 and a median of ￥45.

Drug	Number	Expenditure/month（￥）			
		Mean	Median	Minimun	Maximum
Pyridostigmine bromide	763	207.4	150	0	5，200
Steroids	547	51.6	15	0	600
Traditional Chinese Medicine	328	1911.0	1，500	50	30，000
Tacrolimus	278	1366.3	1，200	200	6，000
Azathioprine	117	259.3	108	0	10，000

Drug	Number	Expenditure/month（￥）			
		Mean	Median	Minimun	Maximum
Mycophenolate mofetil	26	1024.9	1，000	350	2，500
Cyclophosphamide	11	512.3	247.5	0	3，000
Cyclosporine	8	873.1	750	100	1，600
Mycophenolic acid tablets	8	948.0	890	330	1，600
Methotrexate	4	52.5	45	30	90

2）Financial burden of drug use-expenditure for single use

Among the 22 patients who had used intravenous immuneglobulin treatment，the single use of the drug ranged from ￥8-53，000，with an average monthly expenditure of ￥25，609.1 and a median of ￥30，000.

Among the 6 patients who had used plasma exchange，the single-use expenditure of the drug ranged from￥8，000-50，000，with an average monthly expenditure of ￥26，817.7 and a median of ￥22，000.

Among the 19 patients who had used rituximab，the single-use expenditure of the drug ranged from ￥3，000-40，000，with an average monthly expenditure of ￥12，497.9 and a median of ￥12，000.

Drug	Number	Expenditure/time（￥）			
		Mean	Median	Minimun	Maximum
Intravenous immuneglobulin	22	25609.1	30，000	8	53，000
Plasma exchange	6	26817.7	22，000	8，000	50，000
Rituximab	19	12497.9	12，000	3，000	40，000
Eculizumb	0	—	—	—	—

3）Medication expenses in proportion to household monthly income

Among all the oral medications， the expenses oftraditional Chinese medicine/proprietary Chinese medicine had the highest proportion to the patient’s personal or family monthly income， with a median proportion to personal monthly income of 43.9%， a median proportion to household monthly income of 26.7%， and a median proportion to household monthly disposable income of 50.0%； this was followed by tacrolimus， with a median proportion to monthly personal income of 42.9%， a median proportion to monthly household income of 21.7%， and a median proportion to monthly household disposable income of 50.0%.

The expenses of steroids had the lowest proportion of the patient's personal or family monthly income，with a median proportion of 0.5%，a median proportion to household income of 0.3%，and a median proportion to household monthly disposable income of 0. 8%；this was followed by methotrexate，with a median proportion to personal monthly income of 2.3%，a median proportion to monthly household income of 0. 8%，and a median proportion to monthly household disposable income of 1.7%.

Drug	Monthly Expenditure（Median.￥）	Proportion to person's income（Median）	Proportion to household income（Median）	Proportion to household disposable income（Median）
Pyridostigmine bromide	150	4.0%	2.7%	6.0%
Steroids	15	0.5%	0.3%	0.8%
Traditional Chinese Medicine	1500	43.9%	26.7%	50.0%
Tacrolimus	1200	42.9%	21.7%	50.0%
Azathioprine	108	3.4%	2.4%	5.2%

Drug	Monthly Expenditure（Median.￥）	Proportion to person's income（Median）	Proportion to household income（Median）	Proportion to household disposable income（Median）
Mycophenolate mofetil	1000	26.7%	21.8%	50.0%
Cyclophosphamide	247.5	6.7%	3.3%	7.8%
Cyclosporine	750	19.9%	10.8%	25.8%
Mycophenolic acid tablets	890	34.3%	23.0%	66.5%
Methotrexate	45	2.3%	0.8%	1.7%

4）Overall medical expenditure in the past year

Among all patients，the median direct medical expense in the past 12 months was ￥12，742.5，the median indirect medical expense was ￥1，700，the median reimbursable expense was ￥0，and the median out-of-pocket expense was ￥14，400.

Medical Expenditure	Median（￥）	Lower Quartile（￥）	Upper Quartile（￥）
Expenses			
Direct	12742.5	3000	30000
Indirect	1700	0	5000
Loss of labor	0	0	1000
Total	16000	4950	39200
Reimbursable Expenses			
Medical Insurance	0	0	3000
Commercial Insurance/Donation	0	0	0
Total	0	0	3000
Out-of-pocket Expenses			
Paid by Person	14400	3650	32615

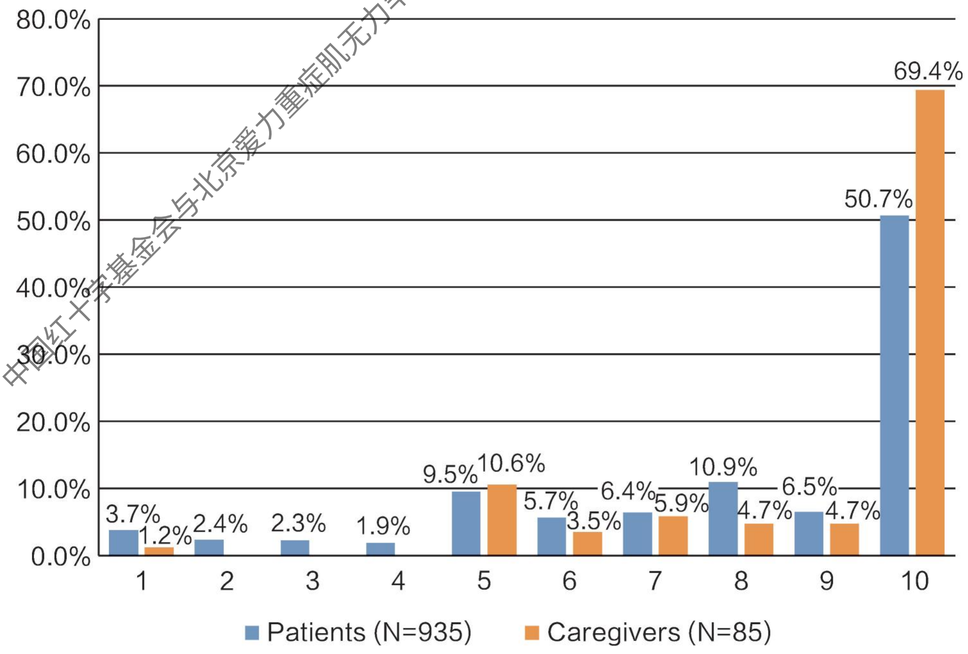
12. Willingness to pay for new drugs-patients vs caregivers

In this survey，935 patients who filled out the questionnaires themselves and 85 caregivers who filled out the questionnaires on behalf of patients were asked about their willingness to pay for new drugs for MG.

1）Urgency for new drugs

This survey uses a 1-10-point scale to measure the urgency of patients and caregivers for new MG drugs，with 1 representing the lowest urgency and 10 representing the highest urgency.

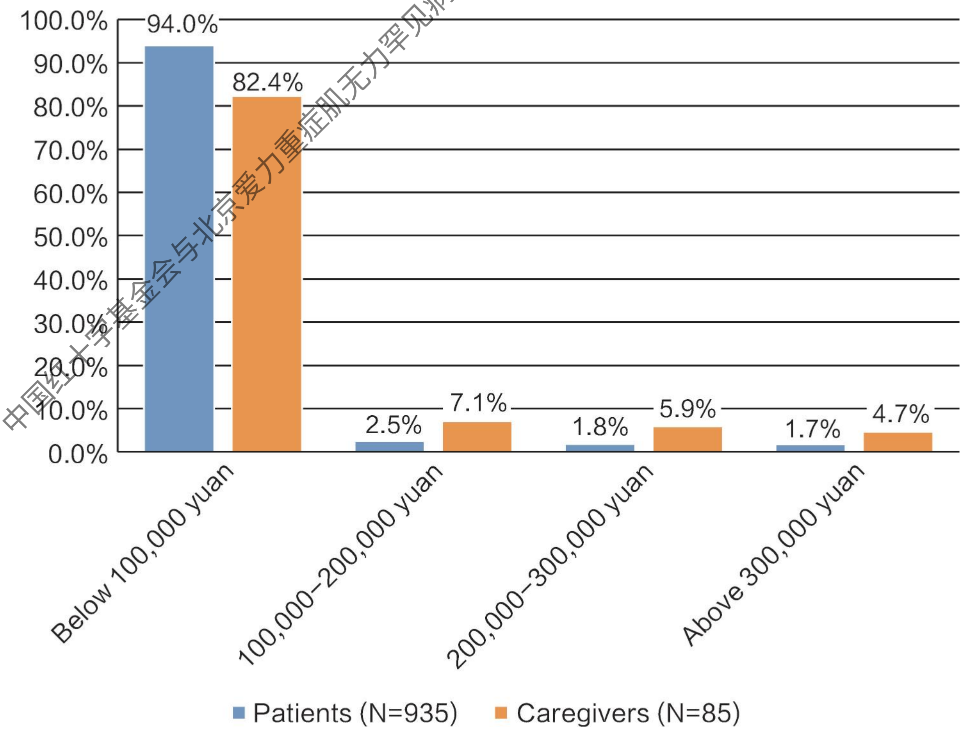
Of the 935 patients，50.7% expressed very high urgency for a new drug（10 points），and 3.7% expressed very low current urgency for a new drug（1 point）. Of the 85 patient caregivers，69.4% expressed very high urgency（10 points），and 1.2% of the caregivers expressed very low urgency（1 point）.



2）Willingness to pay for new drugs

When asked about acceptable prices for new MG drugs，879 patients indicated that the maximum annual payment that they could accept was less than ￥100，000，accounting for 94.0%；23 patients were willing to pay a maximum of ￥100，000-200，000，accounting for 2.5%；17 patients were willing to pay a maximum between ￥200，000-300，000，accounting for 1.8%；and 16 patients were willing to pay ￥300，000 and above，accounting for 1.7%.

Among the caregivers of patients，70 caregivers indicated that the maximum annual payment they could accept was less than ￥100，000，accounting for 82.4%；6 caregivers were willing to pay a maximum of ￥100，000-200，000，accounting for 7.1%；5 caregivers were willing to pay a maximum of ￥200，000-300，000，accounting for 5.9%；and 4 caregivers were willing to pay a maximum of ￥300，000 and above，accounting for 4.7%.



Among the patients who were willing to pay under ￥100，000 per year at maximum，the specific amount ranged from ￥0-80，000，with a mean of ￥122，042 and a median of ￥10，000.

Among the caregivers of the patients who were willing to pay under ￥100，000 per year at maximum，the specific amount ranged from ￥20，000-60，000，with a mean of ￥150，457 and a median of ￥10，000.

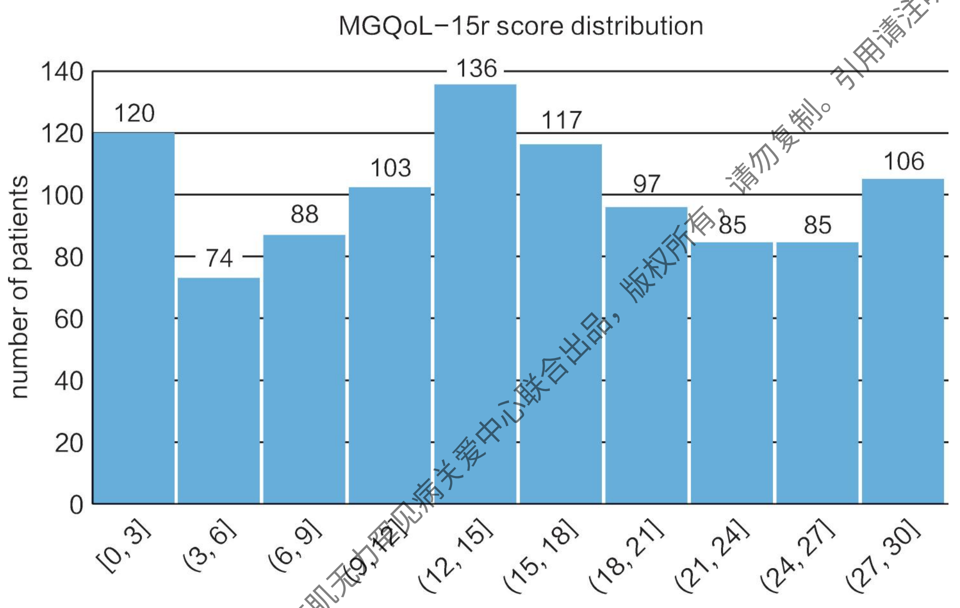
Participant Category	Mean （￥）	Median （￥）	Minimum （￥）	Maximum （￥）
Patients （N＝879）	12204.5	10，000	0	80，000
Caregivers （N＝70）	15045.7	10，000	200	60，000

13. Quality of life，disease impact and social support

1）Myasthenia Gravis Quality of Life Scale（MGQoL-15r）

In this study，the 15-item Myasthenia Gravis Quality of Life Scale（MGQoL-15r）was used to assess the quality of life of adult patients. The scale measures patients' quality of life in terms of mobility（9 items），symptoms（3 items），mental health（2 items），and overall satisfaction（1 item）. The total score ranges from 0-30，with a higher score indicating worse quality of life.

The results showed that among the 1,011 adult patients, the MGQoL-15r score ranged from 0 to 30, with a mean of 15.2 and a median of 15.0. The average score of male patients (15.6 points) was slightly higher than that of female patients (15.1 points), suggesting that the quality of life of male patients was slightly worse than that of female patients.



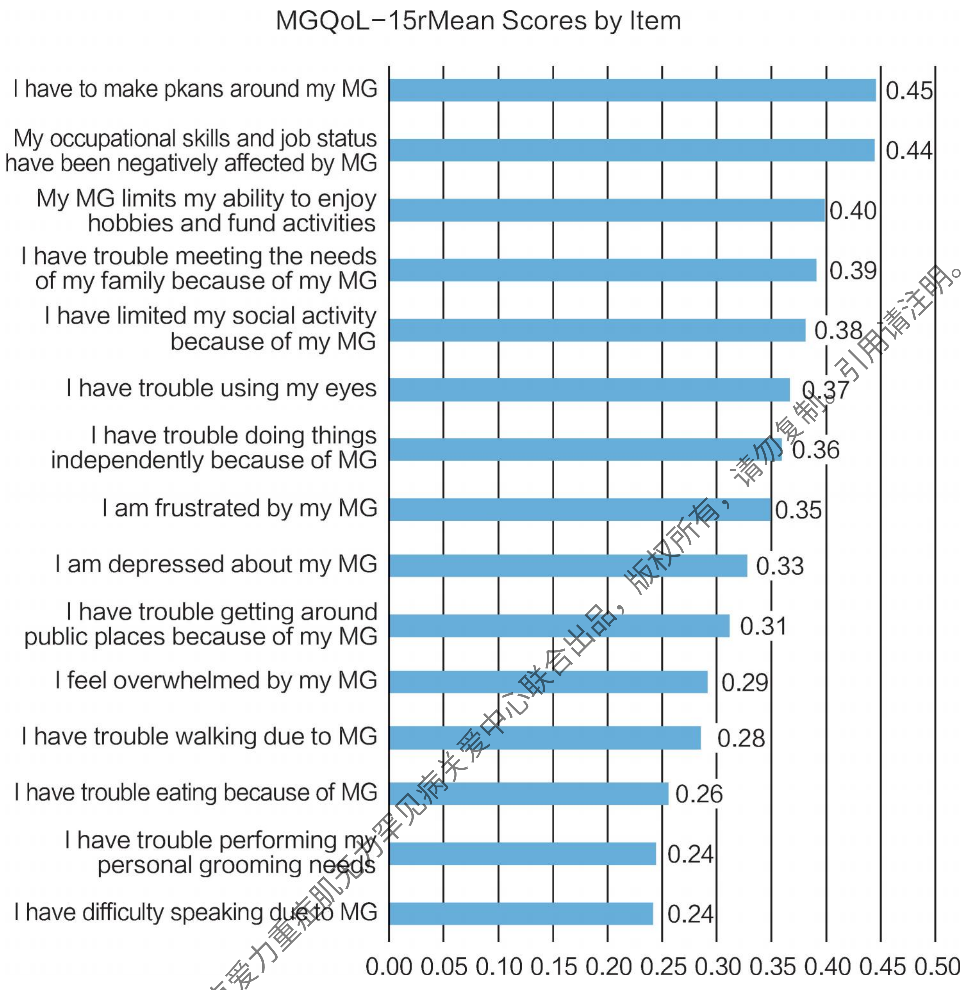
Sex	Mean	Median	Minimum	Maximum
Male	15.6	15	0	30
Female	15.1	15	0	30
Total	15.2	15	0	30

As a reference to some extent，the overall quality of life score reported by MG patients from this survey was lower than the scores reported by patients participating in the “The 2018 National Survey on the Living Conditions of Patients with Myasthenia Gravis in China” and scores reported by patients from “2020 General Social Survey among Patients with Rare Diseases in China”. This indicates that the overall quality of life of the patients participating in this study has improved.

Survey Year	Mean	Median	Minimum	Maximum
2018	15.9	16	0	30
2019	18.4	19	0	30
2022	15.2	15	0	30

Among the MGQoL-15r items in this survey，the items with the highest mean score were “I had to plan around MG”（0.45 points）and “My performance at work or school has been affected by MG，and I am troubled by this limitation”（0.44 points），which suggests that the patients were most affected in these two areas.

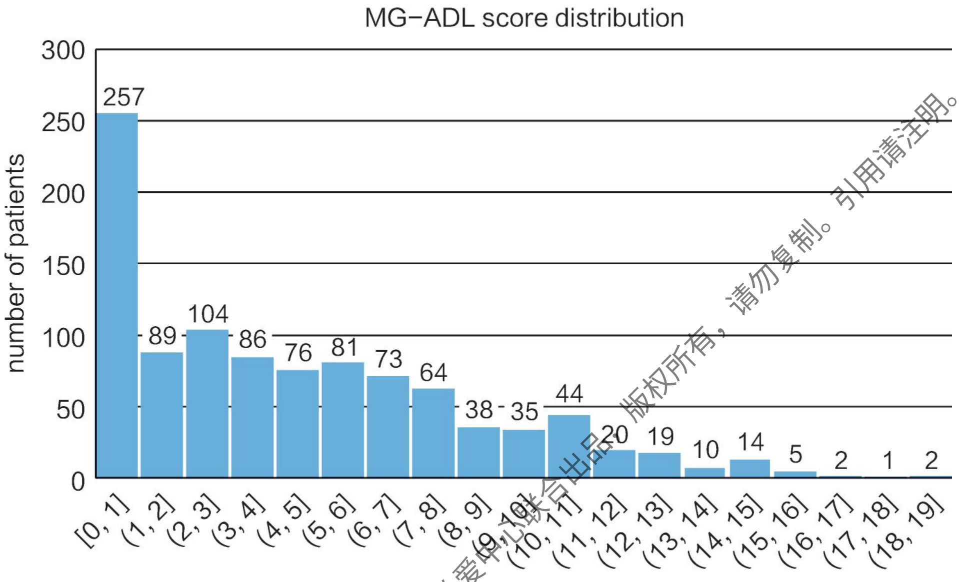
The items with the lowest mean score were “I have difficulty speaking because of MG”（0.24 points）and “I feel powerless to organize my demeanor”（0.24 points），which suggests that the patients were least affected in these two areas.



2）Myasthenia Gravis Activities of Daily Living（MG-ADL）

The study used Myasthenia Gravis Activities of Daily Living（MG-ADL）to assess the patients’ ability to perform daily living activities. The scale assessed the patients’ ability to perform eight activities of daily living such as speaking，chewing，swallowing，breathing，and eyelid droop. The total score ranges from 0 to 24 points，with a higher score indicating lower living ability.

The results showed that among the 1020 respondents，the MG-ADL score ranged from 0 to 19（the highest possible score is 24），with a mean of 4.9 and a median of 4.0. The mean score for male patients（5.3 points）was higher than that of female patients（4.8 points），indicating that the living ability of male patients was worse than that of female patients.



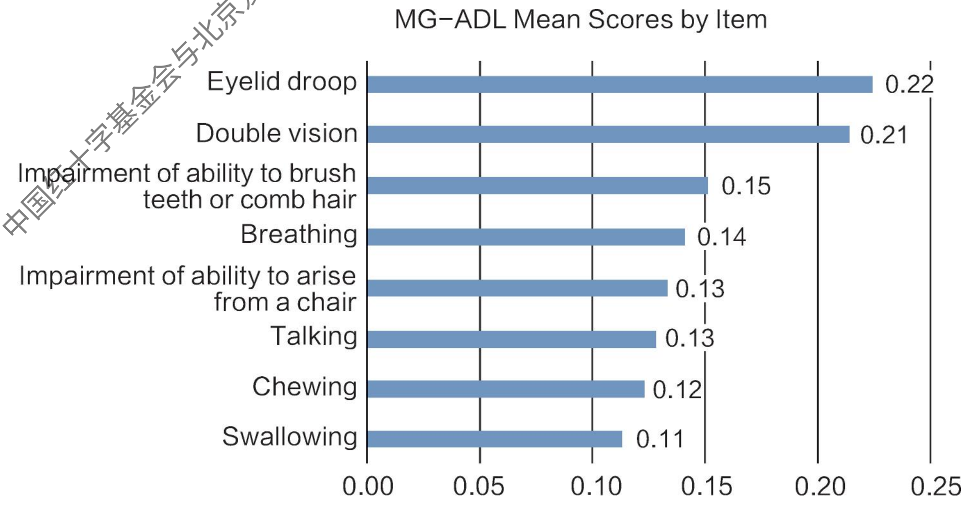
Sex	Mean	Median	Minimum	Maximum
Male	5.3	5	0	19
Female	4.8	4	0	19
Total	4.9	4	0	19

Although it can only be used as a reference to a certain extent，the MG-ADL score reported by MG patients was in general lower than the scores reported by MG patients who participated in the “The 2018 National Survey on the Living Conditions of Patients with Myasthenia Gravis in China” and patients from “2020 General Social Survey among Patients with Rare Diseases in China”. This indicates that the overall daily living ability of the patients participating in this study is relatively good.

Survey Year	Mean	Median	Minimum	Maximum
2018	6.4	6	0	24
2019	6.2	6	0	22
2022	4.9	4	0	19

In this survey，among all the items of MG-ADL，the items with the highest mean scores were “eyelid droop”（0.22 points）and “diplopia/ghost vision”（0.21 points），suggesting that the patients were most affected by these two aspects.

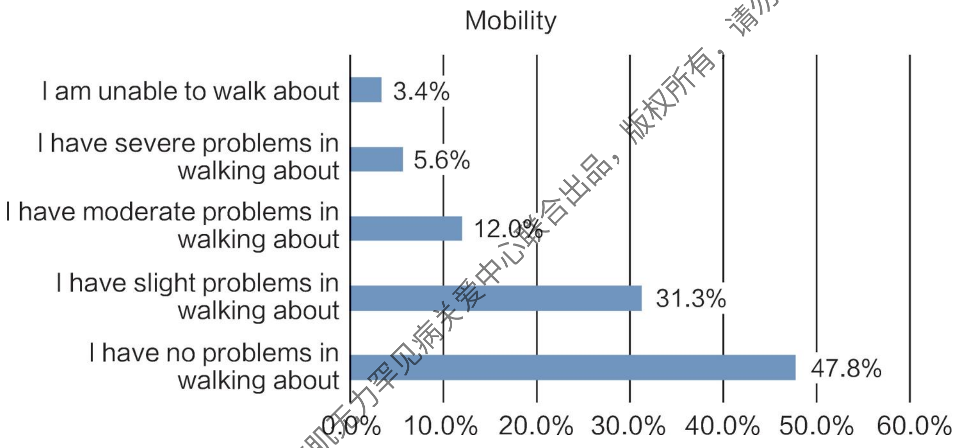
The items with the lowest mean scores were “swallowing”（0.11 points）and “chewing”（0.12 points），representing the least impacted aspects.

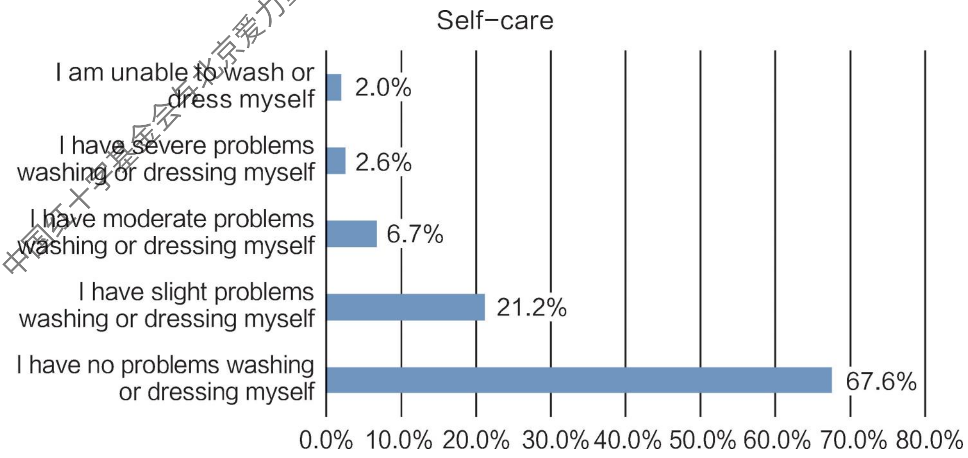


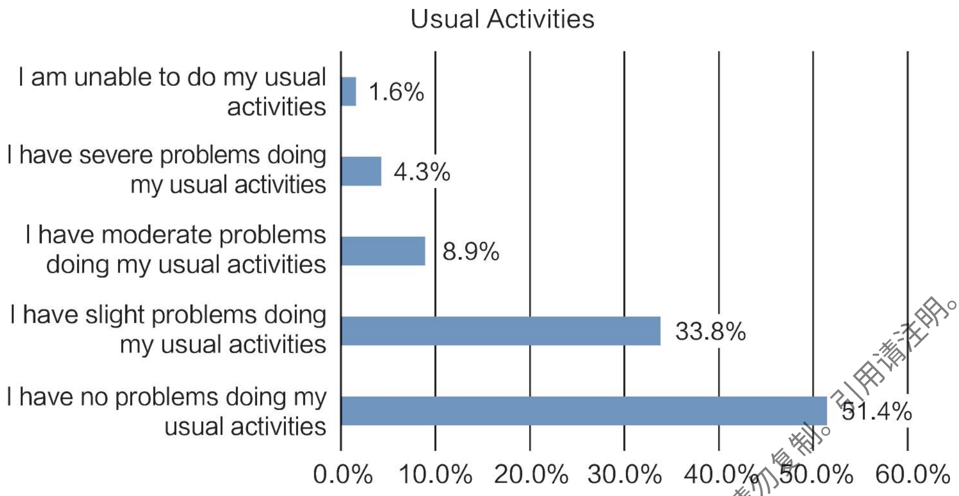
3）Overall Quality of Life-EQ-5D-5L & EQ-5D-Y

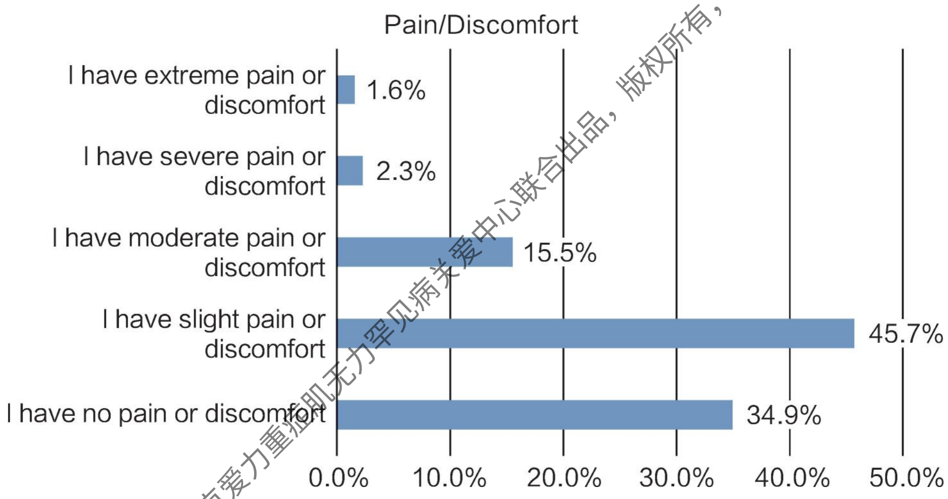
The survey used the EQ-5D-5L scale to assess the overall quality of life of patients aged 13 and above，covering five dimensions including mobility，self-care，daily activities，pain/discomfort，and anxiety/depression.

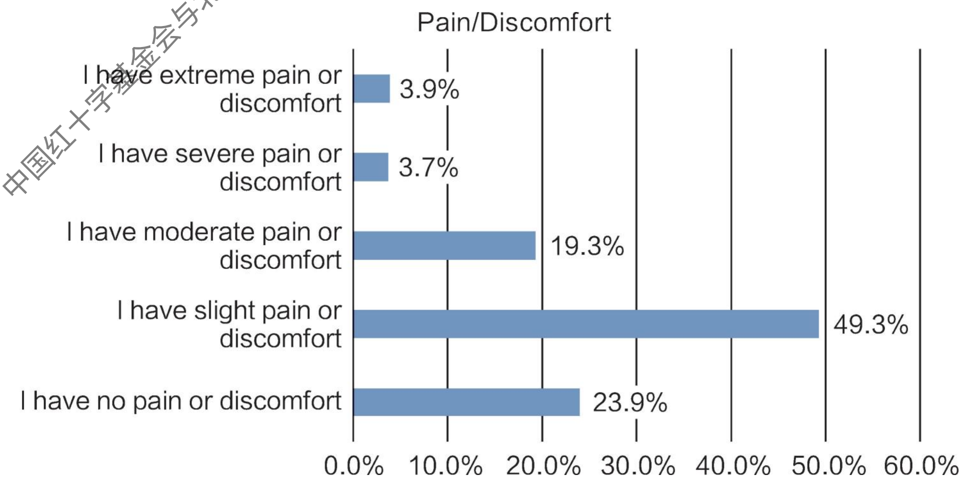
Results showed that about 50% of the patients had no difficulty with mobility，self-care，and daily activities，while about 50% had some difficulty with pain/discomfort and anxiety/depression.





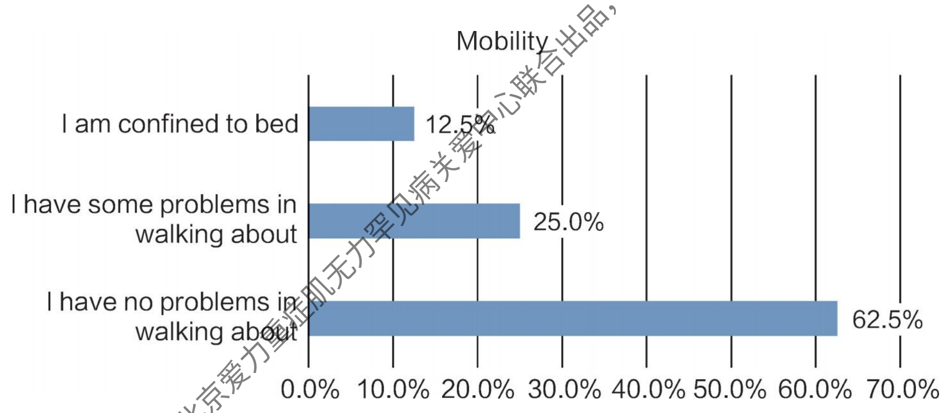

Usual Activities
I am unable to do my usual activities
1.6%
I have severe problems doing my usual activities
4.3%
I have moderate problems doing my usual activities
8.9%
I have slight problems doing my usual activities
33.8%
I have no problems doing my usual activities
51.4%
0.0%
10.0%
20.0%
30.0%
40.0%
50.0%
60.0%

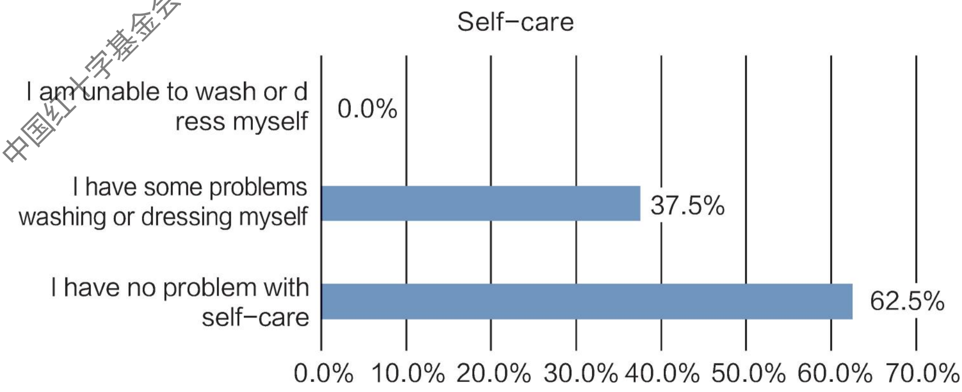

Pain/Discomfort
I have extreme pain or discomfort
1.6%
I have severe pain or discomfort
2.3%
I have moderate pain or discomfort
15.5%
I have slight pain or discomfort
45.7%
I have no pain or discomfort
34.9%
0.0%
10.0%
20.0%
30.0%
40.0%
50.0%

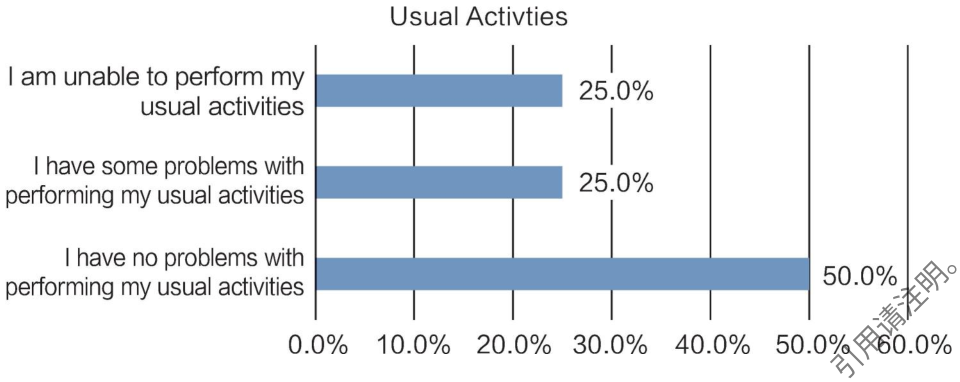

Pain/Discomfort
I have extreme pain or discomfort
3.9%
I have severe pain or discomfort
3.7%
I have moderate pain or discomfort
19.3%
I have slight pain or discomfort
49.3%
I have no pain or discomfort
23.9%
0.0%
10.0%
20.0%
30.0%
40.0%
50.0%
60.0%

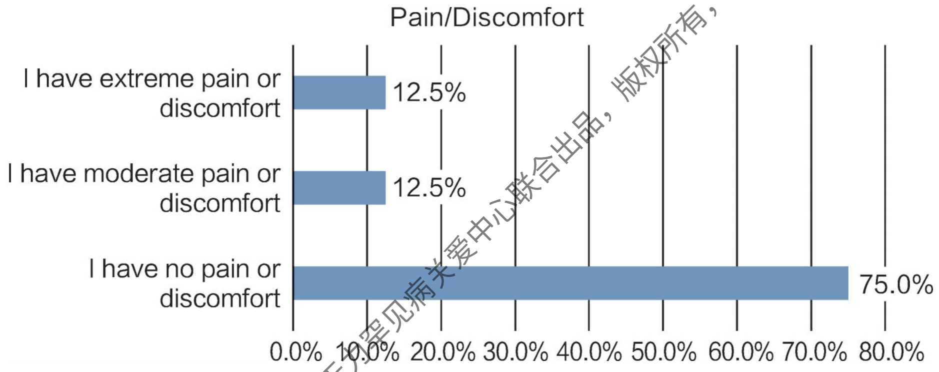
The survey also used the EQ-5D-Y to assess the overall quality of life of patients aged 5-12，which mainly covered mobility（moving around），taking care of himself/herself，and doing normal activities（e.g.，going to school，interests and activity，sports，play，doing things with family or friends），pain or discomfort（such as a headache or an itching），and feeling depressed or anxious.

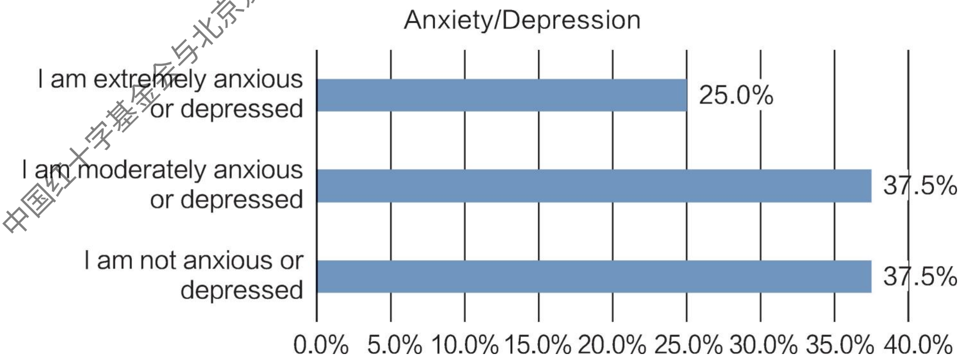
Of the 8 pediatric patients，12.5% had great difficulty with mobility，25% had great difficulty doing things that theynormally do，and 25% felt very worried，sad，or unhappy. However，they had less difficulty caring for their pain or discomfort. No patient had significant difficulty caring for oneself，and 75% had no pain or discomfort.






Usual Activties
I am unable to perform my usual activities
I have some problems with performing my usual activities
I have no problems with performing my usual activities
25.0%
25.0%
50.0%
0.0%
10.0%
20.0%
30.0%
40.0%
50.0%
60.0%

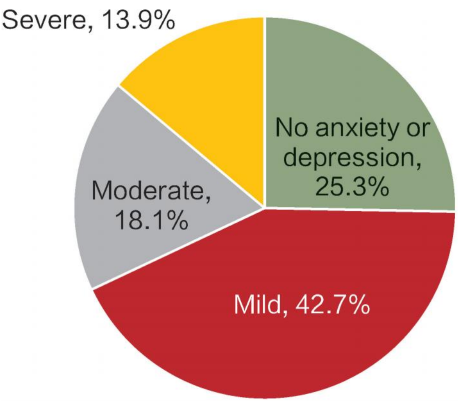

Pain/Discomfort
I have extreme pain or discomfort
I have moderate pain or discomfort
I have no pain or discomfort
12.5%
12.5%
75.0%
0.0%
10.0%
20.0%
30.0%
40.0%
50.0%
60.0%
70.0%
80.0%


Anxiety/Depression
I am extremely anxious or depressed
I am moderately anxious or depressed
I am not anxious or depressed
25.0%
37.5%
37.5%
0.0%
5.0%
10.0%
15.0%
20.0%
25.0%
30.0%
35.0%
40.0%

4）The Four-Item Patient Health Questionnaire for Anxiety and Depression（PHQ-4）

This study used the 4-item Patient Health Questionnaire（PHQ-4）to assess the level of depression and anxiety in adult patients. The scale includes two dimensions：anxiety（2 items）and depression（2 items）. The total score ranges from 0 to 12，with higher scores indicating higher levels of anxiety and depression.

The results showed that among the 1，011 adult respondents，the PHQ-4 score ranged from 0-12，with a mean of 4.7 and a median of 4.0. The mean score of male patients（5.2 points）was higher than that of female patients（4.5 points），indicating that the level of anxiety and depression in male patients was higher than that in female patients. Overall，there were 140 patients with severe anxiety and depression（9-12 points），accounting for 13.9%；183 patients with moderate anxiety and depression（6-8 points），accounting for 18.1%；432 patients with mild anxiety and depression（3-5 points），accounting for 42.7%；and 256 patients with no anxiety or depression（0-2 points），accounting for 25.3%.

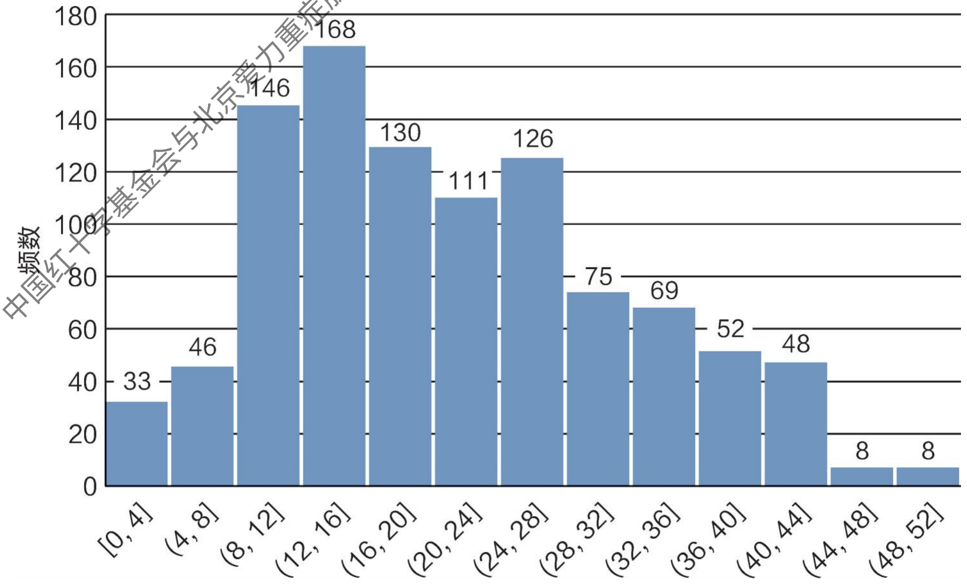


Sex	Mean	Median	Minimum	Maximum
Male	5.2	4	0	12
Female	4.5	4	0	0
Total	4.7	4	0	12

5）Functional Assessment of Chronic Illness Treatment-Fatigue（FACIT）

The study used the Functional Assessment Chronic Illness Treatment-Fatigue（FACIT）to assess the degree of fatigue in patients. The scale contains 13 items that assess the impact of fatigue on the patient's physical，functional，psychological，and social aspects over the past seven days. The score ranges from 0 to 52，with higher scores indicating less fatigue in the patient.

The results showed that among the 1，020 respondents，FACIT scores



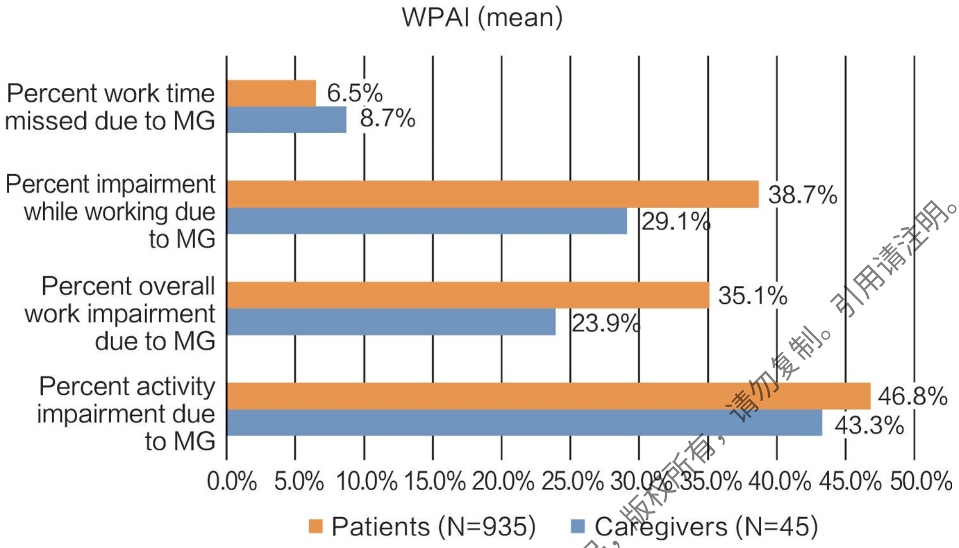
ranged from 0 to 52，with a mean of 21.6 and a median of 20.0. The mean score of male patients（22.8）was higher than that of female patients（21.1），suggesting that female patients had more severe fatigue in the past seven days than males.

Sex	Mean	Median	Minimum	Maximum
Male	22.8	22	0	52
Female	21.1	20	0	52
Total	21.6	20	0	52

6）Work Productivity and Activity Impairment（WPAI）：Patients vs Caregivers

The survey used the Work Productivity and Activity Impairment（WPAI）scale to investigate the percentage of adult patients and patient caregivers currently working who were affected by health problems or caring for patients，that is，what percentage of their work and daily activities were affected.

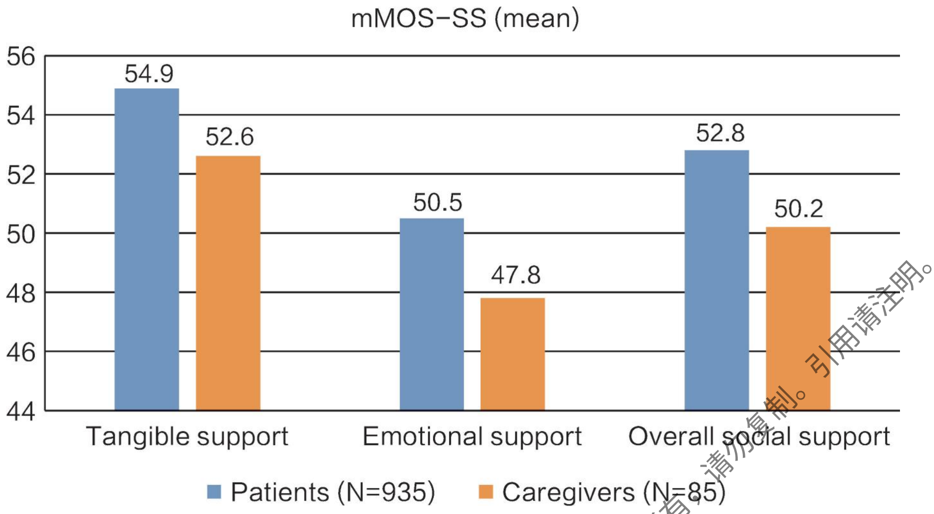
Among the 298 currently working patients，the average absent time due to health issue was 3.6 hours over the past seven days；and among the 45 currently working patient caregivers，the average absent time due to health issue was 6.2 hours over the past seven days. In terms of work productivity，overall work，and daily activities，adult patients were more affected than the caregivers；but in terms of working hours，the caregivers were more affected than the adult patients.



7）Social Support（mMOS-SS）：Patient vs Caregivers

This survey used the 8-item Medical Outcomes Study-Social Support（mMOS-SS）scale to evaluate the overall social support received by adult patients and their caregivers. It mainly measures two aspects：tangible support and emotional support. The total score ranges 0-100，with a higher score indicating higher social support.

Overall，patients'mean social support score（52.8）was higher than the caregiver's mean score（50.2）. The scores of patients in both tangible support（54.9）and emotional support（50.5）were higher than those of patient caregivers（52.6 and 47.8）.


mMOS-SS (mean)
56
54
52
50
48
46
44
54.9
52.6
50.5
47.8
52.8
50.2
Tangible support
Emotional support
Overall social support
Patients (N=935)
Caregivers (N=85)

Part Ⅲ

The Patient Journey of Myasthenia Gravis Patients

14. Introduction to Patient Journey Research

1） Introduction to the stages of patient journey of myasthenia gravis patients

This section describes the MG patient’s journey of seeking care，treatment，and recovery from the onset of the disease to its stabilization. Using 33 patient and family interviews and 7 physician interviews，the report divides the patient journey into the following seven stages.

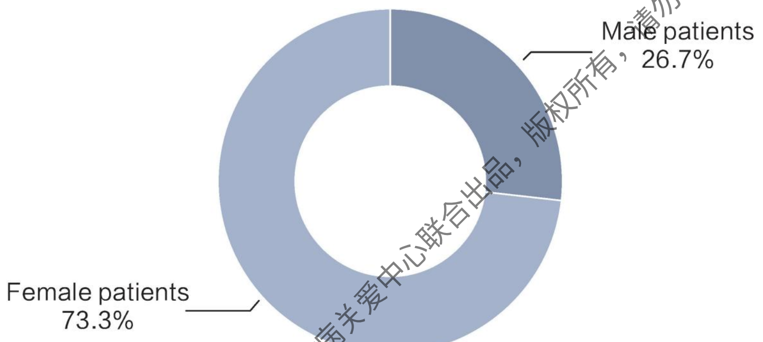
Patient journey	Illustration
Onset	Patients seek medical care based on the initial symptoms of their disease.
Diagnosis	A series of diagnostic procedures of the patient，including the departments to which the patient was first referred and transferred prior to the diagnosis，the diagnostic method utilized and the key factors for the diagnosis.

Patient journey	Illustration
Treatment	Treatment methods used by the patient, including first-, second- and third-line drugs and their side effects, thymectomy and its postoperative effects, as well as other treatment methods such as high-dose gammaglobulin treatment, plasma exchange, traditional Chinese medicine treatment, etc.
Relapse and crisis	Relapses and conditions of danger experienced by the patient during treatment, and their causes and solutions.
Organization Services and Support	Drugs, funding, and other social support received by the patient during treatment and rehabilitation.
Recovery	The patient's strategies and needs in recovery
Current medical condition and other needs	The patient's current disease status and their psychological, social security, employment, and other needs etc.

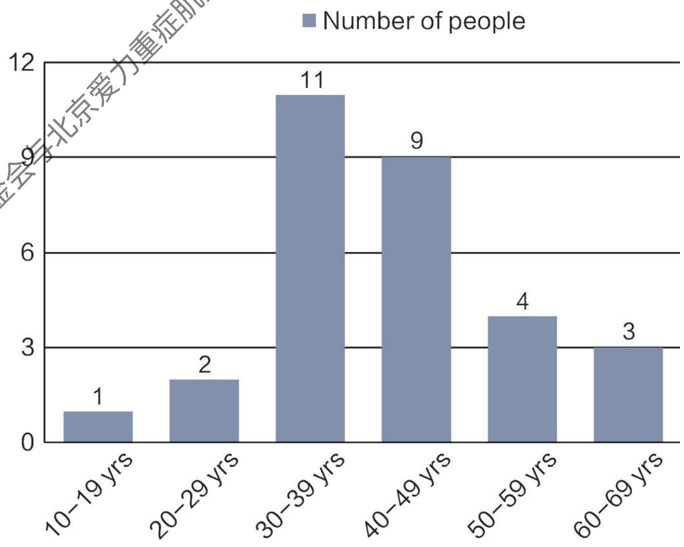
Introduction to the stages along the patient's trajectory

2）Basic demographic characteristics of patients

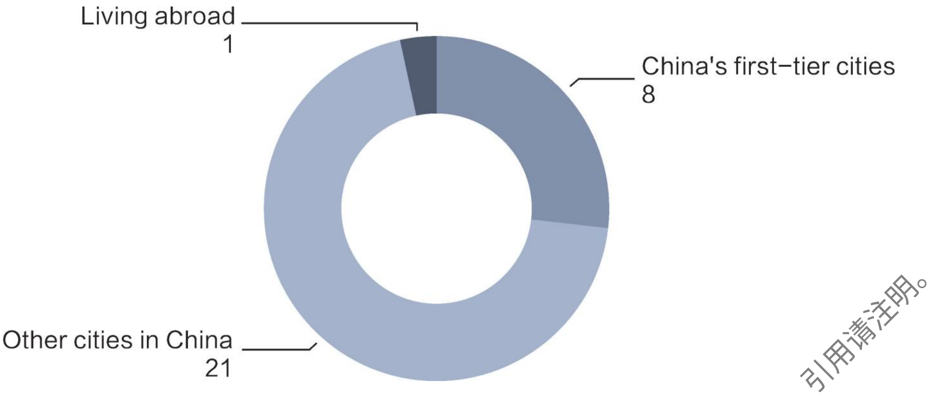
Of the 30 patients whose data were collected in this study, 22 were female and 8 were male. They were aged between 18 and 65.Among them, 8 lived in first-tier cities such as Beijing，Shanghai，Guangzhou，Changsha，Chengdu，and Hangzhou，21 of them lived in other provinces and cities in China，and one patient lived abroad. All of them were gMG patients，and around 70% of them had undergone thymectomy. In addition，86.7% of the respondents in this study could be covered by at least one type of medical insurance including urban workers，urban residents，new rural cooperative medical scheme，commercial insurance or others，though 13.3% of them were not medically insured at all and had to bear all their medical expenses by themselves.



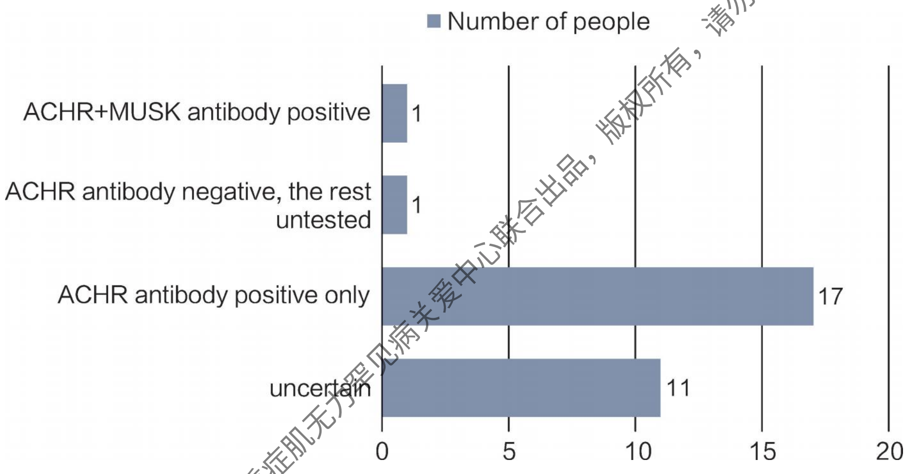
Gender distribution of patients



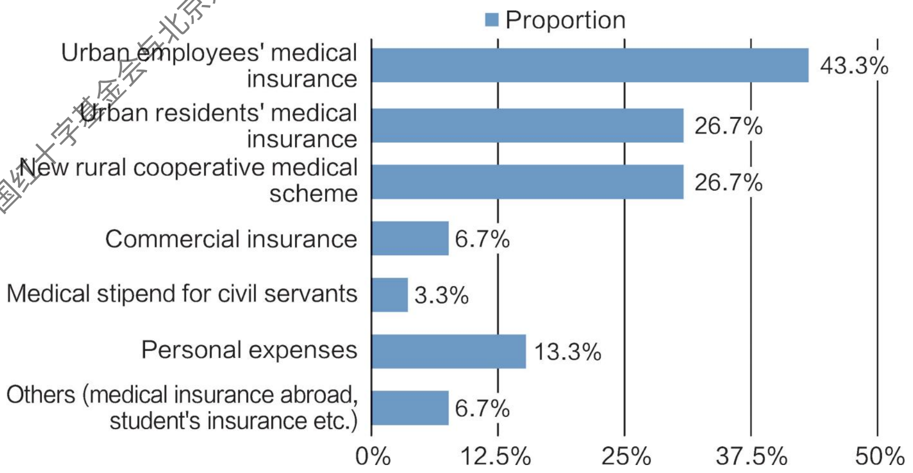
Age distribution of patients



Distribution of patients' place of residence



Distribution of patient's antibody types



Distribution of medical insurance coverage among patients（30 people in total）

3）Distribution of interviews with patients，family members and physicians

	Patient interview				Family interview		
	first-line treatment	moving between first- and second-line treatment	second-line treatment	Refractory	first-line treatment	second-line treatment	Refractory
Beijing/Shanghai/Guangzhou/Changsha/Chengdu/Hangzhou	2	-	3	3	1＊	1＊	1＊
Other provinces and foreign countries	7	2	4	5	1	2	1

Distribution of patient and family interviews

Note：in the family interviews，the family members marked with an asterisk（＊）are those who were interviewed with their patients. Those without an asterisk are those who were individually interviewed.

	Neurology	Thoracic Surgery	Traditional Chinese Medicine
Beijing/Shanghai/Guangzhou/Changsha/Chengdu/Hangzhou	2	1	1
Other provinces and places	1	1	1

Distribution of interviews with physicians

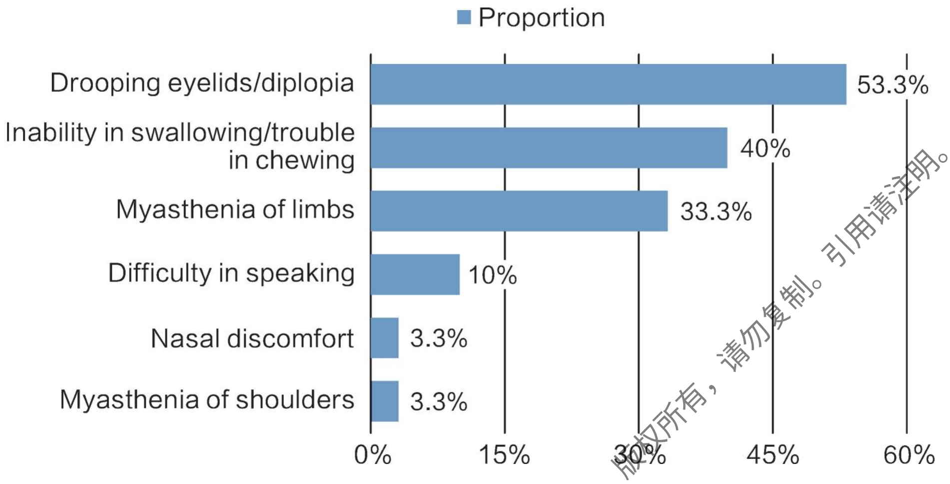
15. Patient Journey

1）Onset

According to the Guidelines for the Diagnosis and Treatment of Myasthenia Gravis in China（2020 Edition），MG may develop in all age groups. Among the patients interviewed in this study，the earliest age of onset was 6 years old and the latest was 55 years old. This section will describe the patient's main symptoms and related understandings and behaviors at the first onset.

1.1 Symptoms

The first onset symptoms of MG vary，among which drooping eyelids or double eyes are the most common. Other symptoms include weakness in chewing，difficulty in swallowing，difficulty in speaking，weakness in walking or raising arms，difficulty in climbing stairs，etc. The distribution of the specific initial symptoms among the patients is shown in the figure below.



Distribution of first symptoms of patients（30 people in total）

It is worth noting that about one-third of patients have a rapid progression of symptoms after the first onset，from a single symptom to a generalized form，or even involving the respiratory muscles.

“In the beginning，I had difficulty chewing and felt exhausted from wearing braces，and then I often felt drained and could barely lift up my hands when combing my hair. Therefore，I had to cut my hair short. Afterwards my eyelids could not be raised，they drooped down after a while. And with pronouncing words. The teacher left heavy loads of homework and gave us lots of materials to recite，after long-time reciting，I could barely pronounce anything.”

——P-XJ-03

“In fact，my onset time came very quickly and only lasted ten days to half a month. When I got into the hospital，my limbs barely had any strength.”

——*P-BJ-09*

1.2 Cognition

Most MG patients and their families do not receive enough attention when the patient first develops the disease. There are two main explanations, the first is that knowledge about rare diseases is not well established at the public level, and people have never heard of this disease; the second is that patients and their families have not yet established a modern consciousness of seeking medical treatment, and they even attribute the symptoms of MG to superstitious elements such as "evil qi", hysteria, or mental illness. For example, many patients will turn to local "prophets" or seek help from clinics without formal medical qualifications rather than going to the hospital for formal examination, resulting in delays to their treatment and deteriorations of their condition.

"(Those prophets) will give you some remedies, similar to taking medicine or likewise, burn some paper, write a talisman for you, and let you drink talisman water after burning it. I followed their instructions but my medical condition hasn't improved at all."

——*P-LN-01*

Although some patients can evidently feel their symptoms of muscle weakness, they have been repeatedly dismissed by themselves or their family members, believing that they are caused only by fatigue or depression, and some family members even think that the patient has engaged in too much suspicion of their disease.

"Because my condition is on and off from time to time, it's easy for me to

despise myself. I have no idea about this disease so I haven't considered seeking medical care from physicians. But when my condition starts to deteriorate，I have no alternatives but to seek medical help. I was diagnosed with depression at the time and was prescribed medicine for depression by the physician for almost a year. But unfortunately，there has been no improvement."

——P-ZJ-24

A small number of patients were able to realize they have MG shortly after the onset，which was mostly a result of the patient's own health and/or internet literacy. For example，some patients indicated that they had acquired the knowledge of autoimmune diseases in junior high school biology classes or through being exposed to MG patients who appeared on TV. Thus，many of them were able to research their condition on the Internet after the onset of the disease and evaluate their medical condition，which led them to eventually seek proper medical treatment in time.

"My onset started in December and I remember it too well. I remember that my hand started to feel a little numb，and then my condition started to deteriorate in February. One day，my left eyelid drooped down and I found it very difficult to speak. Then，I searched on the internet based on my symptoms and found that I may have myasthenia gravis. Therefore，I went straight to the neurology department of a provincial tertiary hospital in Hunan for diagnosis."

——P-HN-18

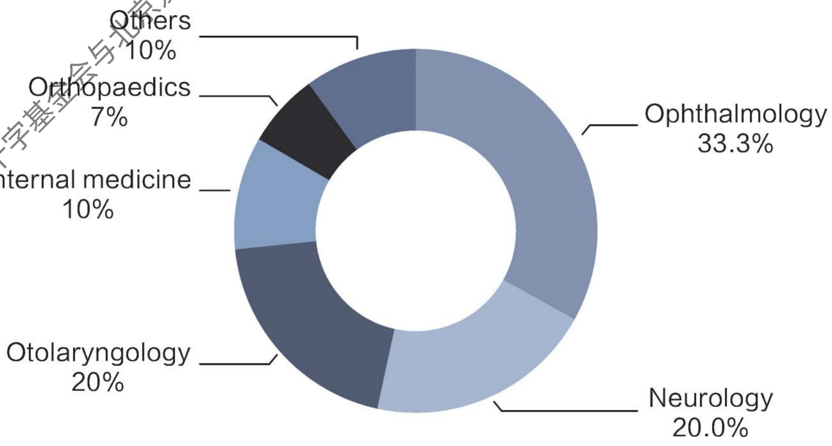
1.3 Behavior

Due to different understandings of the initial symptoms of MG，patients also vary greatly in their medical treatment behavior after the onset of symptoms. Some patients were able to go to the local hospital for medical treatment in time after they or their family members realized the seriousness of the disease. However, some of them spent a long time waiting or observing the development of the disease due to its continuous fluctuation. Although some of them had the awareness of seeking medical treatment, owing to the lack of modern medical knowledge, they preferred to seek help from outpatient clinics or folk remedies without formal qualifications, which may lead to delayed treatment and exacerbation of symptoms.

2）Diagnosis

2.1 First visit to the medical department

As the initial symptoms of patients with MG are dissimilar, the first medical departments in which they found themselves in can also vary. Since ptosis is the most common symptom in patients with MG, one-third of patients sought medical help from ophthalmologists for the first time. Other departments included otolaryngology, neurology, internal medicine, orthopaedics, etc.



Department distribution of patients’ first visit

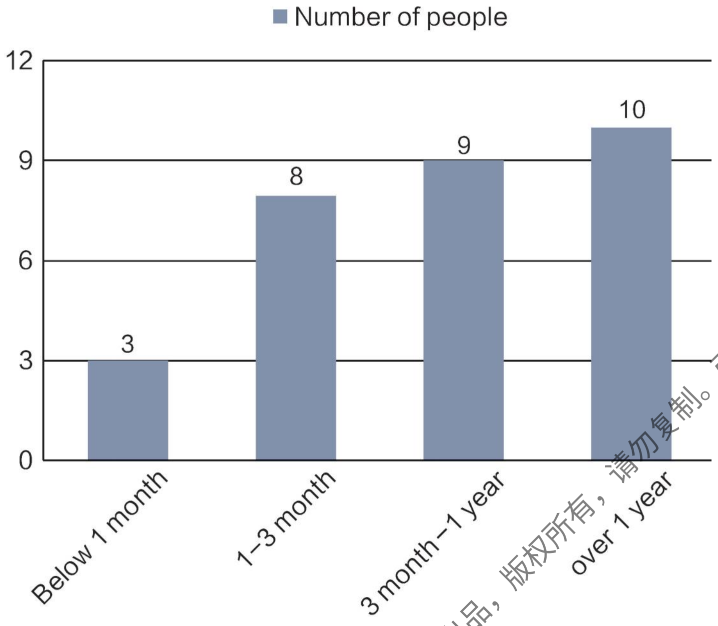
Although around one-fifth of patients can accurately select neurology on their first visit，this does not mean that they could be diagnosed immediately. Due to the unbalanced level of medical resources between various provinces，cities and regions in China，there were still many neurologists who did not understand the disease of MG which led to misdiagnosis.

*“In the beginning，I went to the * * * hospital in Zhengzhou and was transferred to the neurology department. Fortunately，as I had learned that the problem of nerve conduction in the thymus may cause this kind of weakness in my biology classes，I was able to self-diagnose. Then I told my parents so and let them take me to the neurology department. On arrival，the doctor therefore asked me to perform a few movements and so on，and eventually prescribed some nutritional supplements for me like calcium gluconate without much examination.”*

——P-XJ-03

Transferal between departments

Among the MG patients interviewed in this study，only 16.7% of them were accurately diagnosed after one visit to the doctor，and the other 83.3% of the patients had to be referred to other departments many times before coming to the right department for accurate diagnosis. The diagnosis is particularly difficult among patients from rural and underdeveloped area，most of them had to wait at least one month for an accurate diagnosis to be made from time after the first onset，and one-third of them waited as long as a year.

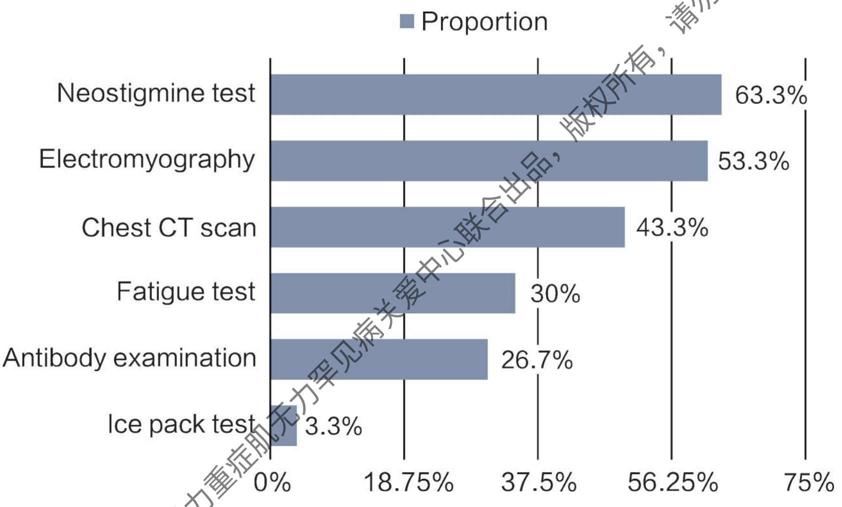


Distribution of the time taken for patients from onset to diagnosis

It is worth noting that there are some key factors that are conducive to timely diagnosis and treatment, including: 1) the ability to be referred to neurology by an experienced ophthalmologist or otolaryngologist; 2) the increase in public awareness and knowledge of this disease; 3) in cases where the patient has an acquaintance who is a neurologist or from any related departments, the ability to visit or be referred to the neurology department. For patients who cannot be diagnosed in time, the common obstacles to diagnosis include: the limited supply of local medical resources, failure of timely referral to other departments such as ophthalmology, otolaryngology, internal medicine; patients or their families' failure to pay sufficient attention to their medical condition; or seeking help from alternative medicine clinics first, resulting in delays to diagnosis.

Diagnostic method

The diagnostic methods of patients withMG vary in many ways. Two-thirds of them were diagnosed by the neostigmine test — that is，test results can be negative or positive by observing the improvement of muscle strength after intramuscular injection of neostigmine methylsulphate reagent. Other diagnostic methods include electromyography，serum antibody examination，chest CT scan，fatigue test，etc.

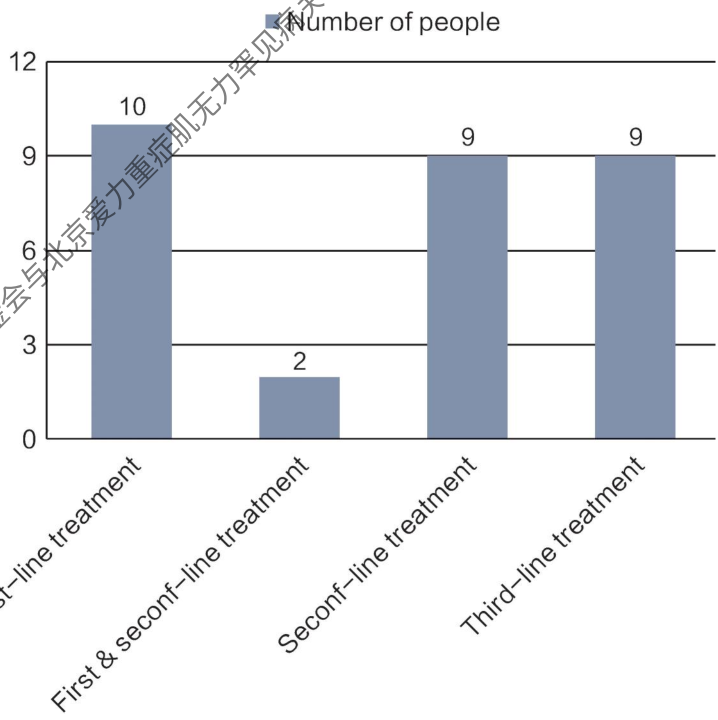


Distribution of patient diagnosis methods

3）Treatment

3.1 Drugs（medication effects，whether the medication was prescribed by the doctor，whether follow-up visits were made regularly，drug side effects）

Drug therapy for MG is divided into three categories：first-line，second-line，and third-line therapy. Common first-line drugs include cholinesterase inhibitors （pyridostigmine bromide），corticosteroids，and azathioprine （AZA），among which pyridostigmine bromide and corticosteroids are the most commonly used drugs among the patients interviewed in this study. Common second-line drugs include mycophenolate mofetil（MMF），cyclosporine，and tacrolimus. Common third-line drugs include cyclophosphamide，methotrexate，rituximab，eculizumab，and targeted therapies. In this study，patients who were treated with monoclonal antibodies or targeted therapies had all been exposed to such drugs by participating in clinical trials of new drug development. Some clinical trials were recommended by the patient's attending doctor，and some patients learned about the trials from patient organizations or related public websites. The distribution of first-line，second-line and third-line treatment among the patients interviewed in this study is shown in the figure below.



Distribution of first-，second-，and third-line treatment of patients

The effects of different medications are related to a variety of factors，including the patient's own antibody type，whether the patient was "refractory"，the patient's own medical compliance，and the degree of communication with the physician. It was found that some of the interviewed patients did not follow the medical instructions from physicians，and their medical condition was therefore worsened.

"My younger brother and sister at home say that taking too much of these steroids is not good since these medicines have side effects. So I stop my medication without asking the physician. Why should I ask the physician? I have given up all of my hope. Then my symptoms start to deteriorate."

——P-LN-06

It is worth noting that patients' medical compliance is related not only to personal health literacy and medical knowledge but also to other structural factors. For example，many patients needed to work outside home for a long period of time because of their poor family economic status，so they were unable to visit the physician regularly resulting in a recurrence of the disease. For example，patient P-NMG-07 went to work outside the home after finishing junior high school and first developed symptoms while working. After being diagnosed，he followed his relatives from Inner Mongolia to Henan to work，and thereafter returned to Baotou，Inner Mongolia，and moved around to Baotou and Hohhot. In each place，the patient looked for a local doctor to reformulate the treatment plan，which also led to the recurrence of his condition many times.

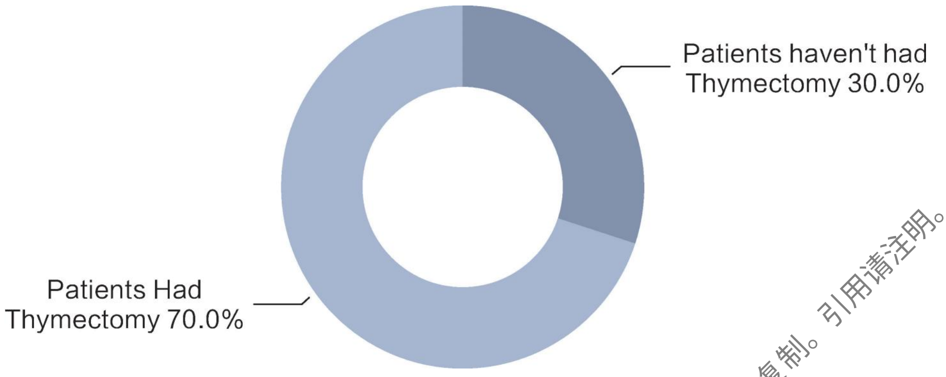
The patients interviewed also mentioned the side effects of the drug as a major source of concern for them. For example，some patients were afraid of taking steroids，because steroids may cause side effects such as weight gain，centripetal obesity，increased blood sugar and blood pressure，osteoporosis，and femoral head necrosis.

> *“Interviewer：What’s the main reason you don’t want to take steroids?*
>
> *Patient：After eating them for a long period of time，my face became swollen.”*
>
> *——P-BJ-12*

Owing to the differences in the quality of medical services across different regions，not all patients were able to take calcium and bisphosphonates drugs to alleviate the side effects from taking steroids replacements drugs. Some patients therefore exhibited symptoms of femoral head necrosis. When they stopped taking their medication，the conditions of their disease deteriorated. In addition，patients also mentioned that the side effects of pyridostigmine included diarrhea，stomach cramps，etc.，that azathioprine may cause bone marrow suppression，liver damage，etc.，and that the side effects of tacrolimus include leukopenia.

3.2 Surgery

Thymectomy is one of the common non-drug treatments for MG patients. According to the “Guidelines for the Diagnosis and Treatment of Myasthenia Gravis in China（2020 Edition）”，MG patients with thymoma should undergo thymoma resection as soon as possible，and most non-thymoma MG patients can also undergo thymectomy to relieve their symptoms. Of the patients with MG interviewed for this study，70% underwent thymectomy. The patient’s thymectomy methods include transsternal surgery，thoracoscopic surgery，and robotic surgery with the da Vinci surgical system.



Distribution of the proportion of patients who underwent thymectomy

（30 patients in total）

The time to effect of thymectomy surgery varies. Most patients reported significant improvement after thymectomy. As one patient stated：

“After the minimally invasive surgery，my medical condition improved 80% from before. Before the surgery，it was difficult for me to talk and walk. Talking with others for more than five minutes frustrated me and I drooled a lot when speaking. In the worst case，I could barely open my eyes. After the surgery，I felt amazing，and the effect has been significant.”

——P-LN-06

However，some patients indicated that thymectomy was not particularly useful. After the operation，the condition of MG did not improve significantly and side effects were evident. As one patient stated：

“After my thymus surgery，my platelet count began to decrease. Some patients suggest that this is a common reaction. Isn’t the human thymus the largest immune organ in the human body? I tell myself that this is normal. After the operation，I

remained in hospital for about half a month. The physician did not say how thrombocytopenia was caused but did say that my platelet count would be normal again after three or four days. However, it never went back to normal at all.”

——*P-LN-01*

3.3 Traditional Chinese Medicine

MG patients often use integrated traditional Chinese and Western medicine to manage their condition. Commonly used Chinese medicine treatment methods include taking Chinese medicine, acupuncture, use of plum blossom needles and Chinese medicine plasters, etc. The distribution of the number of people who used traditional Chinese medicine treatment methods among the patients interviewed in this study is shown in the figure below. They have different views on the efficacy of traditional Chinese medicine. Some of them think that it has no effect at all and others think that it would reduce the side effects caused by western medicine. Also, there were few patients who relied exclusively on Chinese medicine to maintain a stable medical condition.

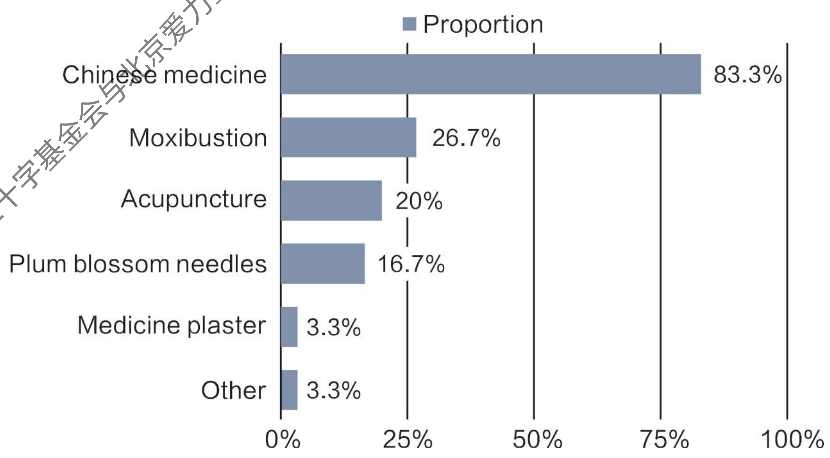


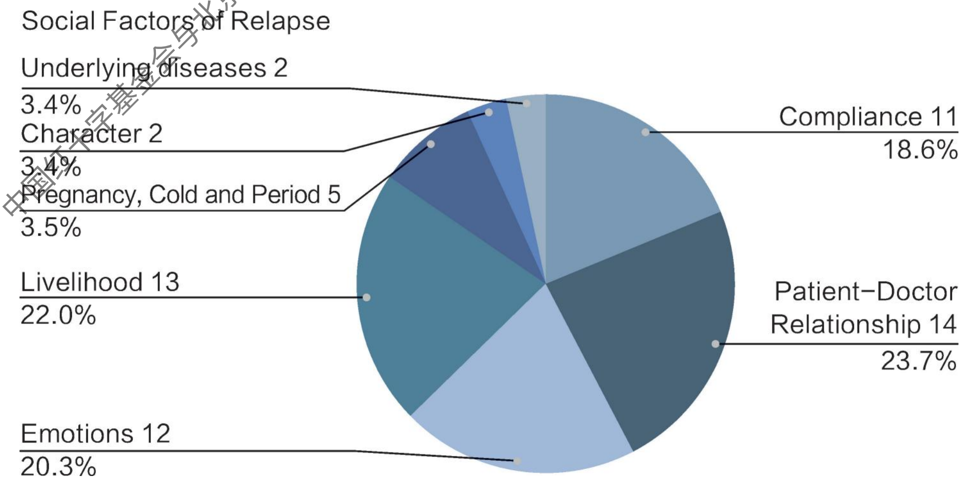
Figure 11. Distribution of the number of patients treated with traditional Chinese medicine（30 people in total）

4）Relapse and Crisis

4.1 Ratio and frequency of recurrence

Owing to the recurring and long-term nature of the disease，patients with MG need to control their condition by strictly taking medicine for a long time and must，at the same time，observe particular lifestyle habits to control their emotions. Among the patients with generalized MG interviewed，28 of the 30 patients experienced repeated episodes of MG and even some serious complications.

Based on the social factors that affect relapse coded in the interview，we roughly identified the main factors that lead to the recurrence of the patient's disease including：compliance（drug out of stock，patients adding or subtracting medicines by themselves，etc.），emotion（influence of disease，family relationship，intimacy，etc.），doctor-patient relationship（no fixed attending doctor，do not believe in Western medicine，etc.），livelihood（economic reasons，physical fatigue，academic burden，etc.），childbirth，flu and menstrual period，personality，underlying diseases，etc.



4.2 Reasons for recurrence and crisis

There are many reasons for relapse. Generally speaking，if the patient does not establish a stable relationship of trust and communication with the physician，they are susceptible to a greater chance of recurrence. Furthermore，inadequate drug supplies and side effects will also lead to relapse. On this basis，physical exertion，emotional influence，and even personality factors can also trigger relapse. In addition，there are some personal physiological factors of patients，such as flu，that may also affect the circumstances. For female patients，menstrual period，pregnancy，childbirth，confinement，etc，may also trigger recurrence and relapse.

The following is an analysis of the causes of recurrence in a typical patient：

P-LN-01，female，diagnosed at the age of 20，has suffered from several relapses since then.

The first relapse was attributed to the unavailability ofpyridostigmine bromide.

The second relapse was attributed to necrosis of the femoris caused by taking steroids. The doctor stopped the drug completely，and the MG symptoms immediately exacerbated；

The third relapse was attributed to the death of her father and the subsequent diagnosis of her mother with diabetes syndrome，and her emotions were impacted；

The fourth relapse was due to helping out at her aunt's family's breakfast restaurant，which was smoky，poorly ventilated，and she felt overworked to the point where she became critically ill and was issued a critical illness notice by the hospital.

Patient compliance

The most common situation is that the patient does not follow the doctor's instructions in taking medications，which leads to the recurrence of the disease. In addition，through communicating with each other，the patient learns about treatment methods and conditions other patients have tried，and accordingly decide to change their medication regime on their own，increasing or reducing the dosage，etc. Furthermore，the patient may refuse to visit the doctor for follow-up consultations owing to the disconnect between the doctor and the patient，which leads to the recurrence of the disease. While relapse might appear to be a problem of patient compliance，other points of trigger also deserve attention.

P-XJ-03，female，14-year-old with symptoms，came to a hospital in Shanghai from Xinjiang for treatment，her symptoms were exacerbated by steroid injection，three months of hospitalizations did not improve her condition，the attitude of the medical staff was awful，and medical expenses costed more than ￥30，000. After seeing the advertisements of a hospital in Hebei and a Putian hospital in Hebei，she tried to seek help from them but was treated as though she was faking her symptoms. Since then，she had lost confidence in medicine，especially in Western medicine. At present，she maintains her condition with pyridostigmine bromide. She lives with her parents，is unable to take care of herself or go out alone.

"I feel that the treatment in Shanghai was a big blow to me，and even going to Beijing for treatment does nothing different."

——P-XJ-03

P-BJ-12，AchR＋MuSK antibody positive patient，onset and diagnosis at 31 years old. Due to the sudden reduction of steroids，resulting in multiple recurrences，he did not trust physicians. He frequently changed his attending doctor and increased or decreased his medication by himself according to his own condition. When the symptoms were more serious，he decided to go back to the hospital in his hometown in Shandong to take gamma globulin and again decided the dosage of gamma globulin by himself.

“The doctors in my hometown’s internal medicine department don’t know much about this disease. They say that if you want us to treat it，then you don’t need to visit us.”

——P-BJ-12

No fixed attending physician

Some patients frequently needed to work across provinces and，therefore，did not have a fixed physician or medication plan. They usually made considerations about whether they needed to go to the hospital based on their own feelings，and because of familial or economic reasons，they manipulated or reduced the number of doctor visits. On the other hand，some patients indicated that they had to passively change the attending physician or reduce the number of follow-up visits because of the difficulty in registering the appointment with the physician and the having to travel long distances for the visit.

*“From the county hospital to the town hospital，at that time it was in Wuhan * * hospital，after visiting the doctor and taking medicine，there was*

not any relief. Later I went to work with my husband at Fujian, I also went through many hospitals when I arrived at Fuzhou, it still did not, the symptoms still did not improve. They kept [referring] me around, thinking that my condition was severe, and then it seemed that they did not have any solutions, and did not tell me how to manage, all they did was push me to this side, push to that side."

——P-FJ-19

Overworked

After the onset of the disease, patients had to make adjustments in both physical and social functioning, and usually during the stable periods do patients choose to return to their family or workplace roles. However, it is also common for the condition to relapse due to continued work or overwork from household responsibilities or child care, which can affect their health status.

"I was working as a cashier in a supermarket and had to communicate with customers a lot. For example, how much money was exchanged, and then I couldn't speak and felt very anxious. At that time, I was very short-tempered. I was very irritated because they couldn't understand what I was going to say, and then I didn't know what was wrong with me. Later, when we ate in the cafeteria, I couldn't eat at all. Then I had to drink soup every day. Even with soup, I would still easily choke. As for doing the laundry, I lost all my strength after washing clothes. For climbing the stairs, I could barely climb them."

——P-FJ-11

Emotional impact

In addition tobeing overworked，emotion is an important factor affecting the condition. In life，patients often relapse owing to the death of family members，discord in family relationships，economic and psychological pressure and other factors that affect their emotions. This is common in both male and female patients.

“My mother-in-law passed away and my uncle also died because of cancer. I felt that my life would always be like this，plagued by trouble. Then my symptoms of thymus hyperplasia became worse.”

——P-LB-06

Flu or female menstrual period

For people with MG，extreme care is taken to avoid catching a flu since it can make the condition worse. Furthermore，menstruation results in changes in the body’s metabolism which may also have an impact on the condition. The interaction between the two factors can also trigger a relapse of the disease.

“I had another period［menstruation，the author’s note］around November last year，but the amount was very small. I thought I was about to enter menopause and felt that my condition was getting better and better. Since I have heard that patients with myasthenia gravis，once they reach menopause，they get better and better. Since whenever a period comes，myasthenia gravis gets worse. Whenever there is menstruation，the condition of myasthenia gravis patients gets worse.”

——P-LN-01

Pregnancy and childbirth

Owing to the physiological changes in women brought about by pregnancy, childbirth and confinement are important challenges faced by women with MG. The relevant literature shows that pregnancy and childbirth may stabilize, improve, or worsen the disease. Among the 20 female patients interviewed, 7 gave birth before onset and 6 gave birth after onset. Among the patients who gave birth before the onset of their disease, 6 of them may have pregnancy, childbirth and confinement as triggering factors of their onset. For female patients with reproductive needs, special attention should be given to the management of their pregnancy with the involvement of neurologists throughout the entire process.

P-BJ-09, a housewife, gave birth twice, and the second delivery was changed from vaginal delivery to caesarean section. She wished to have a second child so underwent diagnosis prior to giving birth the second time. She gave birth in the district hospital in Beijing, and was in a critical state three days after.

"When the (steroid) was reduced to two and a half tablets, I was ready to have a second child. At that time, I was fine in the first few months, but my condition deteriorated in the next few months. It was very difficult for me to swallow, so I basically ate egg custard every day. After the next two or three months, I had already added steroids, so I took 6 tablets of steroids, but I didn't get any better. I was tired but didn't visit the physician because I wasn't in any real danger at the time. On the third day after giving birth, I had a crisis..."

——P-BJ-09

Patient-Family Interaction Factors

“Character” （as its literal translation is colloquially used in Chinese） usually refers to an attitude held towards the world around and is usually reflected in behavior. In the process of disease management，patients with stronger character rarely involve their family members in disease management. Such patients tended to omit important things when explaining their illness to their family，friends，and colleagues，and rarely expressed that they needed family members to participate in disease management or for the family to bear responsibility，believing that they can cope with the problem on their own. This may contribute to an increased risk of relapse.

In addition，behind the avoidance of family involvement in disease management，there are also reasons such as insufficient family relationships and family system support. Illness brings a series of economic and psychological burdens to individuals and families. For patients，if they perceive that they cannot get enough support from their family of origin or the family they form，they are often forced to undertake the burden upon themselves to avoid affecting other family members.

Therefore，how families can provide adequate support in their interaction with patients and how to effectively support the interaction between patients and families are topics that deserve further exploration.

“Growing up in such a difficult environment，I’m still strong enough to handle everything by myself and need not show my weakness to my family and friends.”

——P-GD-08

“I’m all on my own and will never let my family help me no matter if it’s walking or combing my hair.”

——P-HN-18

5）Rehabilitation

5.1 Rehabilitation measures

During the interview，many patients have not been to the outpatient rehabilitation department of a hospital or had any appropriate rehabilitation training. In the context of our country，patients are very unfamiliar with the concept of physical therapy，occupational therapy，and speech therapy in Western medicine rehabilitation.

The understanding of rehabilitation relates mainly to the rehabilitation and healthcare measures from the perspective of traditional Chinese medicine，such as Baduanjin，Wuqinxi，and plum blossom needles. This is a result of the promotion of these rehabilitation and healthcare measures by patient organizations，such as providing online training courses，community activities，and mailing rehabilitation equipment. In terms of rehabilitation equipment，a small number of patients in need were equipped with wheelchairs，walking aids，etc. at home，but most patients were not equipped. If there was a recurrence or crisis，it was mainly overcome with the help of family members.

5.2 Rehabilitation needs

When exploring the rehabilitation needs of patients，many patients mentioned that it is necessary to develop rehabilitation course or training for patients with MG. As for the specific needs，it was difficult for them to express.

Due to economic reasons，the income of most patients and their families was mainly spent on visiting a doctor，taking medicine，etc.，so they were willing to spend only relatively little or even none on rehabilitation and other expenses. Those who were willing to spend were mainly concerned about the rehabilitation and health care measures under the traditional Chinese medicine，the effectiveness of the rehabilitation course for MG，whether there will be interaction with the rehabilitation teacher，the interaction between patients，whether the rehabilitation course could help patients to develop long-term habits，the frequency and extent of the rehabilitation course，etc.

“The class should be held once a week，and I hope to be taught some Baduanjin movements by the teacher. And it is easier to be self-disciplined when there is a teacher，most importantly，it should be free of charge.”

——P-HLJ-26

“I hope that someone will guide me one-on-one，such as on how to do acupuncture better，or what to avoid in daily life. The cost of less than a few thousand yuan is affordable for me. Daily rehabilitation training or exercise therapy is necessary，but not for everyone. No more than 100 yuan per month，once a month，is good enough.”

——P-HLJ-24

“I hope that patients who attend the rehabilitation course together can communicate with each other while recovering. Less than 100 yuan a month，because in the long run，pricy courses will cost too much.”

——P-BJ-17

“Rehabilitation courses require a long period of time and patience. We should integrate rehabilitation training into our daily life bit by bit so that patients can apply what they have learnt. We can pay, but there are many ways to get information, and some do not cost anything.”

——P-HN-27

“I want to learn plum blossom needling. I hope the teacher can communicate and supervise via video link. No more than 100 yuan a month.”

——P-FJ-11

“I am not very interested in online courses. The online coach cannot adjust my movements. If I do it wrong, it may be harmful.”

——P-BJ-12

“I hope it can provide soothing classes such as yoga and breathing regulation. It’s better to put it into a short video, because everyone’s life is fast-paced. A subscription of one or two hundred yuan a month is acceptable.”

——P-JS-13

“I hope someone could help me find the right exercise for my physical condition, because I don’t know what to do now except walk. I hope that there will be interaction between students and teachers. Less than 500 yuan is acceptable.”

——P-ZJ-28

“The duration can’t be too long, and the strength needs to be acceptable, for example, acupuncturing points and adjusting breathing. It is

better if it is free."

——*P-SD-04*

"I hope that rehabilitation training can promote the development of good living habits of patients and that they can put it into practice. The key is to implement the training for a long time."

——*P-BJ-22*

6) Current illness and social support

6.1 Current illness and psychology

Most of the patients were in a stable condition at the time of their interviews. However, like the volatility of the disease itself, patients also had psychological expectations for relapse and being in dangerous condition. In terms of self-care, except for a few patients who needed the care and support of their family members and could not take care of themselves completely, most patients could live and travel normally, but they could not engage in tasks that were too labor-intensive.

In terms of psychology, patients faced relatively common patterns of psychological burden. Owing to the changes in physical, family, and social roles brought about by the disease, patients generally suffered from anxiety, guilt, depression, and other negative emotions, which ran throughout the entire journey of the patient. The main manifestations were anxiety caused by not finding the cause before diagnosis, hope and despair caused by the ups and downs of the condition during the treatment process, among which the doctor's communication, adjustment of their own roles, and the burden of the disease were all triggers.

"I was very desperate and frustrated. At the time, I went back to my family when I was only 25 years old! My family gave up on me."

——*P-LN-06*

"Maybe there are many patients who feel the same as I do, full of guilt and self-loathe..."

——*P-SD-04*

6.2 Self-response

Identity adjustment

Changes in the body after onset of MG impacts the patient's original social role. Before the disease, they were a father, a mother, a husband, a wife, or a child, but MG affected the performance of their original roles and identities. The resulting identity adjustment was a process most patients encountered. This kind of adjustment was due not only to the changes in physical strength, but also in the powerlessness associated with the identity of the "patient" that impacted the possibilities of happiness and satisfaction as a "mother/father/husband/wife/child" would otherwise have. This would bring embarrassment and regret to the patient. This kind of re-coordination and transformation was not only a situation that patients needed to face individually, but also required the roles and functions of other family members to change accordingly.

"When I was seriously ill, the impact was severe. I even had difficulty

walking. Once, I attended my daughter's parent meeting during my fluctuating period, I couldn't even go up the steps in the auditorium, and, at that time, every step seemed so very far away. Then all I could do was to ask the teacher to help me. From my appearance, my symptoms seemed to be unobvious unless they manifested physically."

"If my condition is stable, I will regularly hold the child continuously, but even so my family members kept reminding me not to hold him if I don't have enough strength."

——P-HN-18

In addition to adjustments to their role within the family, patients also had to adjust their role in the workplace. Family members were often more understanding and tolerant of the patient's changes in family role, but the patient's workplace was more uncertain. Usually after onset of MG, patients would implement coping strategies in the workplace environment, such as communicating with the supervisor/manager to adjust the job position, seeking the understanding of the leaders or colleagues, or adjusting the nature of the work, etc.

"Interviewer: I see, so it means that everyone may know your physical condition, but they did not adjust your position, and some strenuous work won't be assigned to you.

Interviewee: Yes, although there is no transferal, but in that job, everyone will take care of you, you know, no one will say that if I lift 100 pounds, you have to lift 100 pounds, no, there is no such situation. He said that you can do what you can and whatever you want."

——P-LN-06

However，we also regret to see that such adjustments in the workplace are not always successful. Many patients，especially women，patients with low education level，and those with serious medical conditions，risk losing their jobs，and many patients after losing their jobs have lost their ability or confidence to look for re-employment，and therefore are relegated to domestic work at home or engaging in scattered work to earn a living.

“Getting sick has had some impact on my job search，because I used to be a jewelry consultant. I was a SA in gold jewelry，and now I take this medicine and became so fat，it’s difficult for me to find a job again，and then I gained weight so my physical strength is not very good，so now I can only do computer customer service and so on，and the income has reduced a lot.”

——P-HN-16

Life adjustment

In order to adapt to the challenges brought by the disease，patients often adjust their lives in various aspects，for instance avoiding going out alone，adjusting work intensity，being careful not to catch a flu，choosing suitable exercise and rehabilitation methods，etc. Some patients will even adjust members in their social circle of family，friends，and colleagues，so that people around them can understand their circumstance and be more tolerant of them.

“For example，we need to stay low and keep quiet to prevent ourselves from becoming annoying and being isolated by others. We need to act like normal，without disabilities，in order to earn a living.”

——P-LN-06

Future options

The disease also affects the patient’s expectations and choices for the future，and therefore changes their life trajectory. On the one hand，it affects the patient’s personal life，such as their studies，job-hunting，and life partner selection，and on the other hand，it affects the patient’s family choices，including familial economic planning，role expectations，and parenting. The connection between the disease and the patient’s future choice renders the patient passive and helpless. Physical health becomes a priority，and the patients’ and their families are faced with the challenge of adapting and adjusting.

“Interviewer：Maybe you used to think that your career is the first priority，but maybe now your health will come first. Right?

Patient：Yes，because I think ideal physical health is fundamental. The purpose of working hard is to have better social security，not to make loads of money，and more importantly I am able to play a good role as a mother.”

——P-HN-18

6.3 Family coping

After the onset of illness，in addition to challenges faced by the patient，owing to the chronic nature of MG and the requirements for disease management，the patients’ family also needs to cope with the disease in caregiving，economic and emotional aspects. At the same time，the way the family responds to the disease is very important to the patient. A positive response will be very helpful for managing the disease，but a negative response will affect the control and recovery of the disease.

Care support

Owing to the gradual development of symptoms since the onset of MG, physical functions of patients are often limited, and symptoms begin with dysfunctions such as ocular myasthenia, ptosis, diplopia, dysarthria, etc. Before a diagnosis is made, patients often go through a long and complicated journey to seek medical treatment, which requires a great amount of care and support from family members. The process of treatment, surgery, and rehabilitation after the diagnosis also requires great effort from them, especially from the primary caregivers.

"Over the past seven years, I have taken care of her alone. I am also worried about the opinions of my daughter-in-law and son, but she is my daughter. Now I am sick, and I have suffered from diabetes for a few years, so I can't take care of her anymore. Her daughter has grown. Now it's her daughter who actually has to take care of her, so she helps her take a bath and do other things."

——P-HN-30

"At that time, I had to ask for leave from work to take care of (my wife, the author's note). On and off, she has been sick for almost half a year. I told my supervisor directly that my wife was ill, and then I took leave to take care of her. She was seriously ill and needed help with everything, like going to the bathroom, taking a shower, etc, since it was inconvenient for her to move."

——P-HN-18 Husband

Financial support

The most direct impact of MG on the family is the increase in the family's financial expenditure on healthcare，especially for juvenile patients who do not have much income themselves or adult patients who do not have a stable source of income. In addition，because most MG patients are women，before the disease，some female patients were mainly housewives，who took on the role of caring for the family and had no stable income. After the onset of MG，they would need financial support from their spouse or even their wider family. For adult male patients，they may have been the main source of income for the family before the onset of MG，and after onset，other members of the family continue to rely on their financial support which has now been reduced. It is important for the family to adjust to the financial situation of the patient and achieve mutual understanding，thus，the patient can avoid further psychological burden from financial difficulties.

"My father was sick in 2000.It was easy for me to borrow money from others as I have the ability to repay the debt. However，when I got sick，no one wanted to lend money to me as I have no ability to repay the debt at all. Now I mainly rely on my father's pension which is about 3000 yuan per month."

——P-LN-06

"Since my sister is sick and would otherwise need money for people to take care of her，therefore，my two sisters stayed at home to take care of her，and my brother went to work at other provinces to earn more money."

——P-BJ-09 family

Emotional support

As mentioned earlier，the disease can have a great impact on the patient’s psychology，which is often lasting owing to its chronic and fluctuating nature. Emotions such as anxiety，guilt，and depression brought on by illness run throughout the patient’s journey，and family members are often also affected by the patient’s mood. However，it is crucial for the patient’s family to provide the patient with appropriate emotional support，as this will not only help the patient face the disease courageously，but also support the patient in facing other subsequent challenges in life in the future.

“My sister and her husband are extremely nice and show a great emotional support to me!”

——P-LN-01 Auntie

7）Patient organizations and local patient communities

Patient organization

After being diagnosed with MG，most patients join a patient group through doctor referral，patient recommendation，online search，or recommendation from friends and relatives，and contact Beijing Aili Myasthenia Gravis Care Center through online links to QQ and WeChat groups to establish contact with other patients，thus receive relevant services of varying degrees. From the website of Aili，we can see that they provide services and projects in the areas of counseling and companionship，rehabilitation and education，doctor-patient communication，assistance and support，integration and employment， community empowerment, and field promotion.

Among the patients we interviewed, many patients gave positive comments on patient organizations. Following diagnosis of the disease, the sooner the patient was able to contact the patient organization, the more likely they would be able to receive the correct diagnosis and treatment, avoiding detours. At the same time, when there was a need for drug discontinuation and medical assistance during the treatment process, the patient organization can mobilize stakeholders such as pharmaceutical companies and hospitals to meet the urgent medical needs of patients by speaking up. In addition, the patient organizations also pay attention to the needs of patients in terms of rehabilitation, psychology, employment, etc., and helps patients in need through empowerment and cultivation.

"In my opinion, the psychologists teach us how to overcome our obstacles. After getting sick all by ourselves, wouldn't we all be very pessimistic?"

——*P-BJ-09*

Local patient support

The patients were linked online through the patient organization, and the loneliness and pain brought by the illness that outsiders could not understand quickly found resonated with other patients. In addition to online communication, the patient organization would also gather patients in the same area into the same patient group to facilitate mutual communication and support, and support the formation of offline communities, for example, through the gathering of local patient groups, etc. Due to geographical factors, patients often establish contacts quickly and became familiar with each other. As a result of common medical needs found in the same region, patients can often support each other in diagnosis, treatment, rehabilitation, psychologically and emotionally. The resulting support network built within the patient's local community provides the patient with tangible support in their daily lives.

> *"Because I was carried by that patient at the beginning of the operation, I remember it very clearly.He carried my right hand. His surname was Han, his name was Han * * . He was from Changchun City. I have to remember it for a lifetime (laughs). I was chatting in the Northeast group at the beginning, and the patient's father also helped carry me because I was fat."*
>
> *——P-LN-06*

16. Other issues

1) Doctor-patient communication

Patient's point of view

Through interviewing MG patients and doctors, this study found that poor doctor-patient communication is a major problem hindering patients' treatment and recovery. This section first discusses the reasons for the difficulty of doctor-patient communication from the perspective of patients.

Firstly, many patients said that their doctors failed to explain the pathogenesis of MG to them, that is, why the autoimmune system causes muscle weakness. Likewise, doctors have failed to explain why patients are taking a particular drug, why the dosage is increasing or decreasing, why the drug is being changed, or why they are being treated with thymectomy, C-globulin pulses, or plasmapheresis. This may be a result of the busy consultation system in the hospital, patients generally feel that they have too little time to meet with the doctor during each visit, and they cannot ask the doctor about the disease management process or understand it in-depth. On the other hand, the patient himself admitted that he did not have sufficient medical knowledge and "didn't know what questions to ask the doctor". This communication barrier hinders patients' understanding of their own disease, and in part results in some patients not paying enough attention to disease management and failing to follow doctor's instructions for treatment.

"It seems that there is a barrier between doctors and patients, and how they study this disease seems to be void of communication with patients."

——P-XJ-03

Secondly, some patients do not have enough trust in doctors, which also leads to difficulties in communication between doctors and patients. There are complex reasons behind the lack of trust: because most patients do not have an attending doctor who has long-term knowledge of their condition, they seek a doctor at random for reasons such as going out for work, difficulty in registration, etc., so doctors have only limited information about their medical history and past medication. This leads to the fact that some doctors prescribe drugs that are not suitable for the patient's current condition, which leads to fluctuation or even recurrence of the disease, which in turn leads to discord in the doctor-patient relationship. In this case, the patient either avoids communication with the doctor as much as possible or thinks that the opinion of a single doctor does not have sufficient authority，and selectively adopts the doctor's prescription according to his/her own understanding.

"I felt that the treatment in a ＊＊ city was also a big blow. Later，I gave up，and I felt that it was impossible to find a good doctor. I thought I might just stay at home. Then I learned Chinese medicine by myself on the Internet，and then bought some Chinese medicine myself. If you go outside，no one is able to cure it，and you need to spend a lot of money."

——P-XJ-03

Researcher："Then why didn't you listen to the doctor's advice and change to azathioprine or Tac?"

Interviewee："Because after that，I don't believe in the authority of doctors. Generally，if I have to make a major drug adjustment，I will ask a few more doctors. When I'm done，I will put the opinions of these doctors together. That's who I am，that's my personal habit."

——P-LN-06

Finally，the lack of patient compliance will strain the doctor-patient relationship. Patients often describe that they always feel that the doctor is "blaming" themselves for failing to take medicine on time. Such a register of blame will further make the patient become afraid of communicating with the doctor and reduce the number of follow-up visits，causing a vicious circle.

"For example，the ＊＊ doctor has a bad temper. He is kind of coercive，he just wants me to say what he wants to hear from me，and the conversation is not very pleasant. He always thinks that I am disobedient. Later，he didn't care about me."

——P-FJ-11

Doctor’s point of view

During our interviews with doctors from neurology，thoracic surgery，and traditional Chinese medicine，we found that doctors also believed that doctor-patient communication was an important factor affecting patient compliance and disease management. During the research process，we found that，from the perspective of doctors，the main factors affecting doctor-patient communication include the following factors：

Firstly，the interpretation of MG in Chinese and Western medicine is completely different. Western medicine may explain the disease pathogenesis，pathogenic factors，diagnosis and treatment，medication explanation，etc.，while traditional Chinese medicine tends to explain from the perspective of yin and yang. Perhaps these two explanations construct the patient’s understanding of the disease.

Secondly，the patients are usually referred from ophthalmology，thoracic surgery，and other departments to the neurology department，which usually involves the patient experiencing a tortuous path of misdiagnosis and mistreatment that before obtaining correct diagnosis and treatment. Throughout the process，the sense of trust in doctors as a whole is affected. The increase in the number of patients starting out in the correct department in recent years is conducive to the building trust between doctors and patients.

Finally，the communication attitude and skills between doctors and patients are very important. Many doctors interviewed mentioned that patients’ compliance depended on equal communication between doctors and patients，whether they can explain to the patients their physical condition in plain language，and whether doctors can explain the testing and treatment plan in detail to the patients，etc.，these are the key factors affecting patient compliance.

We found that due to the chronic nature of the disease and the difficulty ofmaking appointments for follow-up visits to the doctor under China's healthcare system，many patients usually think that they can become an expert after a long-time dealing with their own disease and no longer needed regularly follow-up visits. However，doctors believe that at present，it is difficult for patients to regularly report their conditions to doctors，for example，due to difficulties in making appointments，long travel distances，and segmentation of diagnosis and treatment. Thus，doctors cannot fully grasp the patient's condition and response to medication.

2）Drug Purchase Channels

There are different channels for purchasing medicines for MG patients. Most patients have the experience of obtaining medicine dispensed from the hospital or pharmacy. However，there are times where the medicines are out of stock or are too expensive，thus，patients need to go out of their way to buy the medicines they need and at an affordable price. The interviews found that the methods of purchasing medicine mentioned by the patients are as follows.

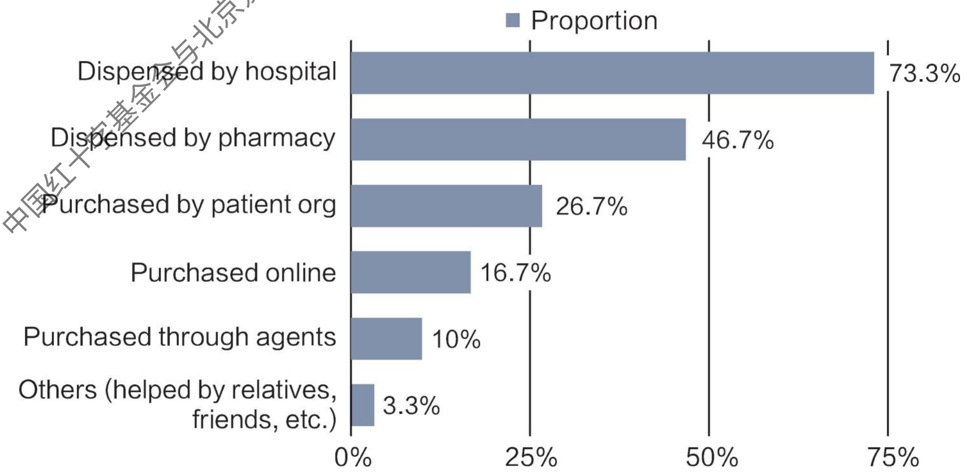


Figure 12. Distribution of drug purchase channels for patients（30 people in total）

It is worth noting that many patients mentioned that pyridostigmine bromide has been out of stock in many areas in the country for several times, so they need to find new ways to buy medicines. In addition, third-line drugs such as tacrolimus are financially burdensome for most patients. Multiple patients mentioned that they compare drug prices from various sources and choose the ones that are cheaper and "look legitimate". There is no way for patients to verify whether the drugs purchased online or through pharmaceutical agents are genuine, which may represent some hidden dangers in the pharmaceutical market.

3) Telemedicine

Due to the difficulties in making appointments, seeking medical treatment in other places, and finding a suitable attending doctor, many patients eventually try and become receptive to the method of finding medical treatments online to help themselves. Among the patients interviewed in this study, 36.7% of the patients have used or are using platforms such as Good Doctor for Internet medical treatment. However, these patients believe that Internet medical treatment can only be used as a means to assist the diagnosis, rather than being solely relied upon.

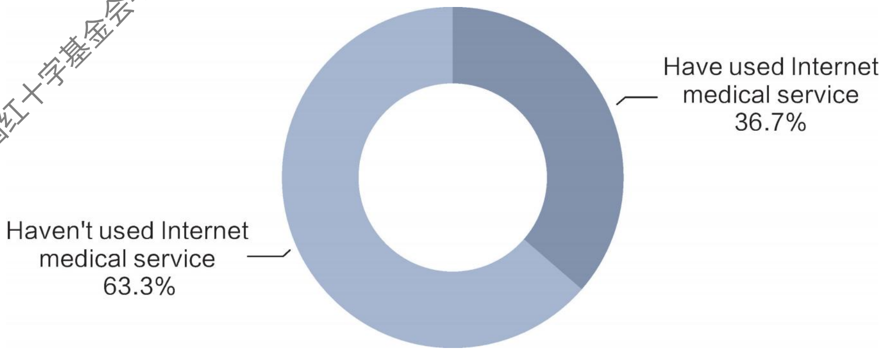


Figure 13. Distribution of patients' Internet medical service experience (30 people in total)

Most of the patients who use some form of telemedicine recognize the benefits it brings, such as being able to make appointments easily, consult famous doctors conveniently, avoid the trouble of traveling for medical treatment in different places, and making it easier to ask doctors questions. However, some patients mentioned that remote consultation is more expensive than hospital consultation. At the same time, compared with Western medicine, traditional Chinese medicine needs to "see, hear, ask, and know", so telemedicine still cannot replace on-site diagnosis and treatment required in traditional Chinese medicine.

"The benefit of telemedicine is that you can ask many times. In fact, when you go to the hospital, you will be charged fifty to sixty or one hundred yuan to make an appointment with a specialist, and then sometimes the doctor will just give you a few words and you're done. In contrast, I can ask more questions on the internet, that is, doctors on the Internet can go through your medical record and tell you something."

——P-JS-13

" I used the Good Doctor website once. I don't remember exactly what happened at that time, maybe it's because the disease has recurred. Consulting on Good Doctor is quite expensive. After I registered, he called directly. To be honest, it did not solve anything at that time. Even if you said a few words on the phone, he couldn't draw a conclusion for you, he couldn't prescribe medicine for you, and he couldn't fully understand you."

——P-HN-27 Mom

4）The illness impact on fertility

Female patients with MG can become pregnant and give birth normally，but unpredictable factors in these processes have an important impact on disease management and the management of labor during pregnancy. As mentioned earlier，of the 20 female patients we interviewed，seven gave birth before the onset and six gave birth after it. Among the presentations of patients who gave birth before the onset of the disease，we believe that the triggering factors of their onset may be related to the changes in physical conditions brought about by pregnancy，childbirth，and confinement. Among them，there are also female patients who have found themselves in dangerous conditions because of the pregnancy.

There is a gap between obstetricians' knowledge of MG and its management，and that of a neurologist and thoracic surgeons' knowledge of obstetrics. Although we found from the doctors' interviews that many domestic hospitals with experience in MG have noticed the reproductive needs of female MG patients and the resulting disease management challenges，and that some doctors are advocating multidisciplinary management，such as a hospital in Hunan，there is still a lack of awareness for lower-tier hospitals where grass-roots patients often seek medical treatment.

5）Refractory type

Although there is no research on this matter yet，this report hopes to provide some discussion on the concept of "refractory" MG. This study considers "refractory" to be an inherently controversial concept. For physicians，"refractory" MG patients have a more detailed medical definition：（1）inadequate response to conventional therapy；（2）in the absence of clinical recurrence or the need for continuous salvage therapy（such as intravenous injections），the inability to reduce immunosuppressive therapy；（3）severe or intolerable adverse effects of immunosuppressive therapy；（4）comorbidities that limit the use of conventional therapy；（5）the presence of MG even during treatment. For patients，however，“refractory” is more of a socially constructed concept. During the interviews，many patients mentioned that the reason why their disease could not be treated repeatedly was that “I am a refractory type”. However，most of these patients were only AchR antibody positive，not a refractory type according to the medical definition. The reason for the recurrence of the patient’s condition lies more in the challenge of achieving stable treatment regime brought about by his/her disadvantaged socioeconomic conditions. Therefore，the report advocates that the medical and public health fields should pay attention not only to the “refractory” patients defined by clinical operations，but also to such socially constructed “refractory” concepts and to devise for patients appropriate policy and social security support，helping them in treatment and recovery more effectively.

Part Ⅳ

Current status of diagnosis and treatment of myasthenia gravis from the doctor’s perspective

17. Doctor Interview

The seven senior doctors and experts we interviewed have extensive experience in the diagnosis，treatment，and research of MG. We have learned that although the level of awareness and technical skill of the diagnosis and treatment of MG among the grassroots doctors in our country is constantly improving，they have expertise over the symptoms，diagnostic methods，detection methods，and medication basis of MG that can help patients avoid detours during the treatment process. The next challenge is to improve knowledge on such matters as medication，surgery，and symptom subdivision. Further，it is more difficult to distinguish between ocular muscle type and the generalized type of MG.

We know that MG consists of only a small part of the textbook training of clinicians. Therefore，in the process of clinical training，it is very important for doctors to be trained and versed in respect of MG. We face the challenge of standardizing and improving the level of related diagnosis and treatment through on-the-job training and multidisciplinary cooperation.

In addition，doctors and patients perceive the role of TCM differently. Most doctors think that Western medicine is the means of diagnosis，treatment，and rescue，while Chinese medicine is to control and balance the amount of side effects from Western medicine. Patients serve to expand the role of traditional Chinese medicine in the management of MG，and are even “eager to mix up the doctors in an emergency”. This is a very special and interesting phenomenon in the context of our country，which deserves further study and discussion.

Part Ⅴ
Summary

18. Research summary

In this study，sequential mixed methods were adopted，and 1020 valid questionnaires were collected through quantitative research mainly based on cross-sectional survey. Through qualitative research based on in-depth interviews，26 myasthenia gravis patients，7 myasthenia gravis family members and 7 doctors were interviewed to describe the quality of life of myasthenia gravis patients in China and drew the typical journey of a patient with weakness from onset to diagnosis to treatment and recovery，to make the public and the government understand myasthenia gravis disease and the impact of myasthenia gravis on patients，families and society. At the same time，we call on more clinicians to identify and diagnose myasthenia gravis，and hope that more effective drugs will be launched to help patients solve the problems of difficulty in treatment，hard-to-obtain and expensive drug prices. Specifically，we summarize the following points for consideration by stakeholders and the public：

First of all，many patients still face difficulties in diagnosis，and the key

node of diagnosis is whether the patient can be accurately referred to neurology department.In order to facilitate the rapid diagnosis and initiation of treatment for more patients with myasthenia gravis，the awareness in three areas should be strengthened. The first is to promote among doctors with expertise in ophthalmology，otolaryngology，and related fields the knowledge of myasthenia gravis，in order to help more patients who first visit these departments，receive accurate and timely referrals. Secondly，the popularization of knowledge of traditional Chinese medicine should also be strengthened. Therefore，the treatment of integrated traditional Chinese and Western medicine can be carried out more effectively；finally，the relevant public health departments should also strengthen the popularization of knowledge to the public and improve the public's awareness of modern medical knowledge，so that all kinds of patients can find clinic more easily.

Secondly，the key points to prevent recurrence lie in the patient's medication，comorbidities，life and emotional management. In terms of medication，the patient's medication compliance still needs to be greatly improved. To improve this issue，on the one hand，patients' awareness of disease management needs to be improved，and patient organizations should also strengthen medical knowledge education for patients；the trust relationship between doctors and patients is particularly important，and doctors should listen to patients as much as possible. Ongoing support needs to be provided to patients on the basis of feedback，to adjust the most suitable treatment plan. For some refractory patients or patients living in remote areas，the possibility of long-term follow-up by a dedicated person should be explored. For patients in special periods（such as pregnancy and postpartum），the hospital should also strengthen the collaboration between obstetrics and neurology department to help obstetrics professionals provide special support programs for such patients，and actively promote cooperation among multidisciplinary doctors. In addition，the arrangement of patients’ daily life and work，as well as psychological and emotional adjustment，should also be paid enough attention.

Third，many patients with myasthenia gravis are not actual “refractory” patients in the medical definition. The reason why their condition is “refractory” is more due to their disadvantaged social status. To help such socially constructed “refractory” patients obtain more continuous and stable treatment，relevant policy-making departments should support them in social insurance and medical insurance，promote the inclusion of more second-and third-line drugs in the scope of medical insurance，and improve and promote the reimbursement policy of “special outpatient clinics for serious diseases”. Hospitals in various regions should also continue to promote Internet medical solutions to help patients in remote areas and economically underdeveloped areas have more convenient access to high-quality medical resources. In addition，patient organizations should also give more attention and support to myasthenia gravis patients in terms of employment training and assistance，psychological counseling and adjustment，and patient community building，so as to reduce the financial burden of patients and provide patients with comprehensive care.

Fourth，increase doctors’ attention on myasthenia gravis，promote the improvement of the diagnosis and treatment process，and promote the standardization of diagnosis and treatment indicators. In recent years，with the improvement of the diagnosis and treatment process of rare diseases by the group of doctors，if myasthenia gravis patients can accurately find the neurology department of the relevant hospital，the difficulty of diagnosis has been reduced. However，we have also found that due to the different initial symptoms of myasthenia gravis，some patients will go to other departments such as ophthalmology，otolaryngology，ENT，obstetrics and gynecology，and whether doctors in other departments can identify these symptoms and referral is the key for patients to avoid detours and treat their conditions in a timely manner. Therefore，we call on doctors in other departments to have a basic understanding of myasthenia gravis. In addition，as far as neurology is concerned，we found that different types of hospitals have slightly different treatment methods. We call on neurologists to enrich and improve treatment plans according to the diagnosis and treatment guidelines，explain the disease in detail during the treatment process with patients，and strengthen patients' self-confidence in the understanding of the disease and promotes early diagnosis，appropriate treatment for patients，thereby further reducing the disease burden of patients.

Fifth，innovate treatment plans and introduce new drugs. At present，the affordable treatment drugs for patients are relatively onefold，mainly focusing on anticholinesterase inhibitors，steroids，immunosuppressants，etc. Due to the inconvenience of daily use of drugs and various side effects of drugs on the body，the patient's medication compliance is poor（MMAS-8 is only 4.7），which is one of the main reasons for the patient's relapse. Patients and their families are eager to have innovative treatment plans，evaluate the efficacy of new drugs and new treatment plans compared with traditional treatment plans，and adopt better plans for the recovery of patients' physical and social functions to help patients better control the disease and better integrate into society.

Sixth，reduce the financial burden of patients and families through the social security system. The disease has a huge impact on the quality of life of patients. Affected by the disease，most patients cannot work and study normally. About 41% of the patients have no economic income，and 50% of the families have a monthly income of less than 5，000 yuan，thus，the economic burden is heavy. In recent years，a number of appropriate support policies have been issued at the national and regional levels for the diagnosis and treatment of rare diseases. Myasthenia gravis patients need to take medicines for life. The cost of medicines and the availability of medicines will greatly affect the compliance of patients. Therefore，it is very important to adjust the drug use and reimbursement policies of patients in different regions，improve the availability of drug reimbursement through drug and reimbursement policies，and reduce the burden on patients and families through the social security system.

Seventh，help patients and their families improve their quality of life through social support. The disease has a huge impact on the quality of life of patients. Their plans for life，work and study have to be based on the disease. Some patients lose their ability to do things independently，and a small number of patients have difficulty speaking and walking. Only 41.7% of the patients were fully able to take care of themselves. It brings burdens and troubles to the patients themselves and their families. More than 80% of patients will have affected on limb muscles and trunk muscles，which will have a great impact on life；some patients will have affected throat muscles and respiratory muscles，which will increase the occurrence of crises. MG patients should actively intervene，adhere to the treatment，and prevent the recurrence and progression of the disease. In addition，the mental health of patients cannot be ignored. Due to the burden of disease on individuals and families，patients generally have a heavy psychological burden，depression and anxiety are common. Therefore，we propose that the government should introduce more social support measures，including mental health，community care，rehabilitation，etc.，to help patients improve their quality of life.

Eighth，pay attention to the reproductive health of female patients. Among the patients with myasthenia gravis，the proportion of female patients is higher （>70%），and the age of onset is earlier. During the interviews，many female patients mentioned the impact of illness on fertility. Due to insufficient fertility management and control，disease recurrence occurred from time to time. Therefore，whether the relevant departments of the hospital can carry out multidisciplinary cooperation，provide safer and more effective fertility support programs to support their fertility needs，pay attention to the phenomenon of neonatal myasthenia gravis，and more importantly，how to get through pregnancy and childbirth stage while controlling the disease.

Ninth，cultivate civil forces and support the development of patient organizations. The number of rare disease groups is small，and they are faced with difficulties in the accessibility of diagnosis and treatment and lack of social support. Patient organizations have a natural connection with the patient community. In the process of supporting the diagnosis and treatment of rare disease communities such as myasthenia gravis plays a unique role. Patient organizations can not only help myasthenia gravis patients quickly and accurately find the corresponding hospital departments and doctors for appropriate diagnosis and treatment，but also unite the patient community in coordinating doctor-patient relationship and field promotion to carry out corresponding value advocacy and promote the use of patients for the construction of a health care system. In addition，the patient organization provides services for the myasthenia gravis community，such as consultation and companionship，rehabilitation education，assistance and assistance，integrated employment，and community empowerment，which can effectively help patients and their families cope with social difficulties caused by the disease，thereby help the community to better cope with the disease，and refer appropriate resources to help patients cope with the challenges that the disease brings to individuals and families, and better integrate into society. Therefore, the government and civil forces should actively cultivate the patient community, support the development of the patient organization, and introduce relevant resources and training to improve the professionalism of the patient organization service community, so as to further improve the overall quality of life of the rare disease community and more effectively promote a healthy and fair society.

Appendix

Scale 1： The eight-item Morisky Medication Adherence Scale（MMAS-8）

	是	否
1.您是否有时忘记服药?	○	○
2.在过去两周内，是否有一天或几天您忘记服药?	○	○
3.治疗期间，当您觉得症状加重或出现其他症状时，您是否会不告知医生而自行减少药量或停止服药?	○	○
4.当您外出或长时间离家时，您是否有时会忘记带药?	○	○
5.昨天您服药了吗?	○	○
6.当您觉得疾病已经受到控制，您是否停止过服药?	○	○
7.您是否觉得坚持计划有困难?	○	○

	是	否
8.您觉得要按时按量服药很难吗?	○从不 ○偶尔 ○有时 ○经常 ○所有时间	

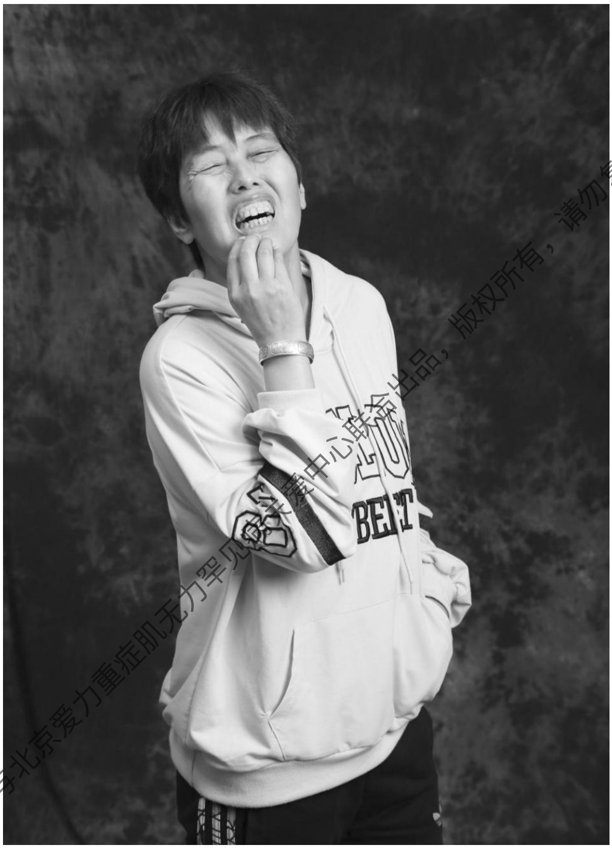
Scale 2：Self-efficacy for Appropriate Medication Use Scale，SEAMS

发生以下情况时，您按时服用或者使用药物的信心是多少？（SEAMS）

	没有信心	有点信心	非常有信心
1.当医生更换你的药物时	○	○	○
2.当你发现重新买的药物与以前的药物在服药方法上有差异时	○	○	○
3.当你每天需要服用几种不同种类的药物时	○	○	○
4.当每天服药次数大于 1 次时	○	○	○
5.当你出门在外时	○	○	○
6.当你某天很忙时	○	○	○

	没有信心	有点信心	非常有信心
7.当药物产生不良反应时	○	○	○
8.当没有人提醒你时	○	○	○
9.当服药程序比较麻烦时	○	○	○
10.当你日常活动计划被打乱时	○	○	○
11.当你不太确信服药方法时	○	○	○
12.当你不太确信在哪个时辰服药时	○	○	○
13.当你患其他疾病时（如受凉或感冒）	○	○	○

Patient Story



Ju Xiuqin, from Heilongjiang province, 31 years with MG. Her parents and younger brother passed away one after another due to illness. She once felt apologetic to her family and regarded herself as a burden of the society. Because of her aunt's care and love and help from kind persons, she gradually regained confidence and was inspired to become a useful person to the society. Now she is a full-time public welfare worker whose main job is to help other patients.

Patient’s words

Zhu Xia，from Liaocheng City，Shandong Province，49 years old，6 years with MG

The pain and despair after illness can slowly subside over time，but the pain of being neglected by society tortures me all the time and makes me suffocate. Strangers，would you give me a smile and a hug?

Cong Shuwen，from Tieling City，Liaoning Province，54 years old，12 years with MG

It has been twelve springs and autumns since I was ill. During these twelve years，there have been sorrows，disappointments，tears and laughter. From being unable to take care of myself completely to now，I have experienced too much. Sometimes when I touch my own head，knowing that I am still alive，tears can’t help but come out of my eyes. It’s really hard to live. From traveling long distances to seeking medical treatment，thinking about being misdiagnosed and detours，and thinking about the pass by death. All these are vividly in memory，which is really frightening. Forget the past. Expect the future. Hope is in front of you. I sincerely hope that better new drugs can be developed，so that more patients can see the light and hope!

Wang Enyu，from Suqian City，Jiangsu Province，30 years old，1 year with MG

In a year and a half，I went from eye muscle type to full body type to bulbar type. A few months ago，I thought I could be like a normal person，but now I obviously feel air leakage every morning. I swallow a mouth of food and then leak a little air like a small fish spitting bubbles，not like hiccups，which is very uncomfortable. I am very distressed，renting a house，medical expenses，work，and often taking sick leave. I am stretched，and my quality of life is getting worse and worse. I'm afraid to think about the future. Now it may be my best state. I hope that the cost of traditional Chinese medicine can be reimbursed to minimize the pain of illness.

Zhao Baoping，from Beijing，45 years old，33 years with MG

Sometimes adversity is a gift that life gives me. I was imprisoned in this square inch of land，without the hardships of making a living，and I didn't have the body and ability to enjoy eating. I think the country can legislate to respect people who are really incurable and in deep pain to implement euthanasia. I'm a childless person who wait until the end of my life. I hope that I can choose to leave decently. When I live，I will enjoy the present moment.

Ren Yuanchun，from Shanghai，45 years old，8 years with MG

I suffered from myasthenia gravis at the end of 2014，and only then did I learn some knowledge about the human immune system. I learned that the long-term sub-health before the illness is a warning to the body. If you improve your diet，lifestyle，and care for mental health early，it is possible to prevent disease from occurring. Even after diagnosis，these aspects should be the direction of long-term rehabilitation of patients. It is hoped that the media will guide citizens to care for healthy lifestyles and mental health.

Wang Shufang，from Suihua City，Heilongjiang Province，66 years old，15 years with MG

How I wish I could no longer use steroids and immunosuppressants. Older people are in poor health and must take steroids and immunosuppressants all year round. I am now suffering from hyperglycemia, hyperlipidemia, high blood pressure, bone degeneration, and kidney damage. But these medicines to maintain the disease are necessary. I really hope that there will be more effective medicines for the symptoms to save children and young patients'life.

Bai Meng, from Xiangyang City, Hubei Province, 35 years old, 6 years with MG

The hardship of life do not only come from the disease. I was still young when I got diagnosis. The main trouble was that in pre-diagnosis days, I had no energy. I want to have some achievements but without enough energy. I always hide myself in case I was being laughed by others. Such days are slowly going away with the diagnosis and treatment of the disease. It was only later that I discovered that those difficult days did not represent all the sufferings of life, nor did the so-called sweetness comes after bitterness. This life is destined to carry forward with heavy burdens.

Li Shuangxin, from Xiangyang City, Hubei Province, 32 years old, 3 years with MG

The pandemic has lasted for 3 years, and I have also been sick for 3 years. From the helplessness at the beginning, to accept the disease and face it bravely now, I have also experienced many difficult nights with the encouragement of family members, the support of friends, the comfort of patients, and the care of doctors and nurses. Although it was unfortunate to be ill, I was lucky to meet many people who helped me along the way. Illness brings physical pain, which we cannot change, but we must always maintain the happiness in our inner world. I hope that our patients will get better and better，and we look forward to more people paying attention to our rare disease group，and we also look forward to more people bravely speaking out for love！

Peng Xie，from Beijing，43 years old，3.5 years with MG

Every patient with rare diseases may have asked the question repeatedly. "Why am I suffering from this disease？" Same was I. Then I realized，"Why can't it be me？" Among all living beings，except for a few treacherous and evil people，who should suffer from this disease？ Sickness is one of the countless accidents and difficulties in our lives，which falls from sky and is caught off guard. People who do not suffer from this disease will also face all kinds of pain and helplessness. Life is like this. No matter how hard it is，you must endure it，because this is your whole life. What we can choose is to live this life well and let ourselves and our family have more good thoughts.

Hu Qian，fromShaoguan city，Guangdong province，28 years old，14 years with MG

Life，

I want to run to you without hesitation.

I want to embrace you with open arms.

I want to look up at you.

I want to call you softly.

I want to smile at you…

These things that are easy in the eyes of others，but I can't do them as I'm suffering，physically and mentally weak. But because of love，every breath is full of courage and effort. I believe that one day，like so many people，we will be healthy and powerful，express our inner joy easily，and no longer receive discrimination from others.

Zhai Xiuzhen，from Hohhot city，Inner Mongolia Autonomous Region，68 years old，17 years with MG

I am a stubborn person. Myasthenia gravis has changed me from an "all-round champion" into a "powerless" person. My mood has reached ice point and I am extremely depressed. Later，I found Aili Myasthenia Gravis Care Center（Aili）. I saw hope when I was with our patients. I felt that it was good to be alive.

Now my condition is getting milder and my health is getting better and better. I don't want to disturb my family. I can completely take care of myself and I can take care of my partner，forming a virtuous circle. Thanks to my family，to Aili，to my myasthenia gravis，for giving me the strength to stand up again，and giving me more energy in my heart!

Zhou Qi，from Heihe city，Heilongjiang Province，53 years old，6 years with MG

I hope that the country will improve the medical security system for myasthenia gravis by including our drugs into the medical insurance list. I hope that patients with myasthenia gravis in remote areas can receive timely diagnosis and treatment，so as to reduce the economic burden of medical treatment in other places. I hope that there will be an employment platform for myasthenia gravis community by providing suitable jobs to relieve the family's economic burden of the patient group. In that case we can draw a lot of color for our own life and contribute to society.

Wang Peng，from Liaoning Province，44 years old，16 years with MG

I have been ill for 16 years. With hospitalized several times in MG crises, my family's financial situation is already difficult. However, the price of the drug " Pyridostigmine" that my fellow patients and I must take every day has been increasing. It is not easy to buy. Besides, even tough immunoglobulin has been included in the medical insurance reimbursement list, why is it not reimbursed when you go to the hospital? One year I was hospitalized in the ICU for more than 10 days.It was really worse for a family with patients all year round. The money for the hospitalization was from my mother who took my three-year-old daughter to borrow from relatives bit by bit. Although I'm an adult, no mother wants her child to be taken away by disease. The child is her sky, and the sky cannot fall down. I hope I can take good care of mother and child in the future.

Huang Xia, from Taizhou City, Jiangsu Province, 44 years old, 10 years with MG.

I always feel that God likes to play tricks on me and joke with me, so I think there is no difficulty that cannot be overcome. Although it takes a lot of hard work and burden to survive, and countless ideological struggles if you choose to persevere, we dare not let anyone know. We are fear of being ruthless, frustrated, despised, neglected, and lonely... We are fear for expressing own sorrow and joy no matter how weak and painful. I can only bury it in my heart. I fear for announcing my condition because I am afraid that I will lose my source of income, and that my employer will no longer hire me. I hope that one day I can take medicine openly. No longer need to pretend to take medicine secretly to maintain. Life can be easier!

Zhou Yanxia, from Wuhan City, Hubei Province, 51 years old, 23 years with MG

I was diagnosed with myasthenia gravis in 1999. In the past 23 years，I have experienced countless times of treatment-relapse-treatment. From the depression，anxiety，and confusion at the beginning to the calm down now，I have come a long way road. I am trying to become a caregiver who can help the patient by using companionship，helping others by self-helping，and living in peace with the disease.

Liu Hongbing，from Linzhou，Henan province，48 years old，13 years with MG

I am a patient who has been diagnosed with MG for 13 years. From 2007 when myasthenia gravis symptoms started to 2009 when I was diagnosed，from when my son was in the first grade to this year when my son graduated from college，I have come all the way for a whole 16 years! I have been given strength by the perseverance and companionship of my family over a long period of time，my own refusal to give up，the donations from Aili and caring individuals，and the assistance of enthusiastic patient friends. Although the moss flower is small，it is still like a peony. Hopefully more and more people from all walks of life will pay attention to myasthenia gravis，offer help，tolerance，and understanding to patients，and help them return to society.

Li Yingjin，from Tianjin，67 years old，8 years with MG

We hope that Aili will be able to help more patients，and that together we can share the passion and mutual help that is conducive to the healing of patients，since we do not only need medical treatment，but also spiritual healing. I also enjoy listening to Aili's lectures on health knowledge and physical therapy methods. Currently，my condition is relatively stable，and I am also benefiting from the treatment and its effects. This is my expectation， demand，sentiment and statement.

Su Xiaozhu，from Maoming，Guangdong Province，66 years old，20 years with MG

Su Xiaozhu is my mother，and I am her son. My mother is an illiterate，rural woman who does not understand Mandarin. Having been ill for many years，she was recently hospitalized and intubated by a ventilator due to COVID-19. Initially，she had recovered，but because the intubation time was too long，the tube was blocked by sputum，and it took more than ten minutes to save her. She was in a coma after being transferred to the ICU. I hope that my mother will wake up soon and leave the hospital safe and sound. COVID-19 is extremely dangerous to MG patients，not only because they are susceptible，but also since most antibiotics cannot be used，and since their respiratory muscles are weak，breathing difficulties are more likely to occur. I hope that the state can introduce some policies to assist MG patients in receiving treatment under COVID-19.

Chen Wenhua，from Chongzuo，Guangxi Province，43 years old，6 years with MG

I suffer from myasthenia gravis，as well as Sjögren's syndrome and anticardiolipin syndrome. I am treated with a combination of Chinese medicine and Western biomedicine. The cost of Chinese medicine is approximately one thousand eight hundred yuan a month. If you add western medicine and other examinations during follow-ups，the total cost is over two thousand yuan per month. Having no parents，no family，no income，and no good job，I rely on my relatives to do some work to support me. Usually when the limit of my chronic disease reimbursement is used up，I have to stop taking Chinese medicine.

There is a limit of 3，500 yuan of my chronic disease reimbursement，but my yearly medication cost is approximately 30，000 yuan. I hope the state can increase the reimbursement for MG so that we can be covered for medication more sustainably.

Zhang Hanxi，from Guilin，Guangxi Province，35 years old，7 years with MG

On the surface，MG patients appear normal，without limb defects，but they are prone to fatigue，and their symptoms often recur. Therefore，it is difficult for us to work full-time for a long period of time. Our quality of life is adversely affected by the financial burden of the medication. If you have a disability certificate，you may be able to enter some companies under the welfare scheme，and the companies will assign you some light work according to your physical ability. You will also be able to enjoy free transportation on subways，buses，and other means of transportation. When you get on the bus，someone may offer up their seat，and you may also receive priority at hospitals and train stations.

Despite the fact that MG patients are permitted to apply for a disability certificate under national policy，most patients are not able to pass the review when their condition is stable after rest. The condition of MG is fluctuating. I am unable to take care of myself when the symptoms occur，and I am not able to even put on clothes or walk on my own. In particular，bulbar type patients appear good，but have difficulty eating，breathing，and are unable to work. Despite this，they were unable to obtain a disability certificate or apply for sick leave. The state should consider the characteristics of MG，evaluate their disability according to the type of disease，and grant disability certificates of level two or higher to patients who suffer from bulbar or generalized myasthenia.

Xie Shuping，from Shenzhen，Guangdong Province，34 years old，14 years with MG

MG is a disease that is clinically curable at best，but not completely curable. Having fallen ill in 2009，I have been taking medicine for a long time. Take pyridostigmine tablets that basically every patient will take for a long time as an example. Only a pharmaceutical factory in Shanghai currently produces these rare disease drugs. A bottle of pyridostigmine cost 26 yuan at that time，but it costs 104 yuan in 2023. Approximately 60 capsules are contained in one bottle. If I take eight capsules a day at most，one bottle can only be used for 7-8 days，and I need four bottles a month. This medicine alone costs more than 4，000 yuan per year. These drug costs do not include the cost of other immunosuppressant drugs. Only a few cities across the country offer chronic disease or special disease outpatient clinics for MG patients，and reimbursement amounts are still very low，making it difficult for most patients to afford treatment. Hopefully，the government will be able to ensure that patients receive their basic medication.

Xue Yazhu，from Leshan，Sichuan Province，52 years old，5 years with MG

Every day I wish that the medical community will develop a cure for MG. At present，my state of health is not very stable，and I am carrying a heavy psychological burden. The biggest lesson I got from suffering from this disease was that the unrelated Aili staff and volunteers are better than my own relatives. Despite never having met them，the positive energy conveyed by love encouraged me to overcome this illness！

Siluhuayu，from Xinjiang Changji Hui Autonomous Prefecture，64 years old，18 years with MG

I look forward to being able to live like a normal person without taking medicine in the not-too-distant future. I can go shopping，participate in any activities，and talk to people all the time… Some people might ask how this is difficult. It is this difficult for me. I has been taking this medicine for ten or eight years，and it relapses after stopping it. After the relapse，it is more serious than before. Sickness comes like a landslide，but goes slowly like spinning silk. Our health improves a little bit every year or two，as opposed to other diseases that improve every day or month. And I am extremely happy even for this little progress，as this is not easy for us. I hope in another year… or maybe in a few years，I won't need to take medicine at all!

Fu Xiuguo，from Zhengzhou，Henan Province，52 years old，10 years with MG

I am from Port Area in Zhengzhou，Henan Province. I rarely go out since I became ill，I do not like to talk，I do not like getting together with friends，I just want to live in my own little world. I cannot overwork because this disease is prone to recurrence. Chinese medicine is not reimbursed in our area，and the price of pyridostigmine tablets have increased from 20 to 30 to more than 100，so I am suffering both from financial and mental hardships. Additionally，I hope that people will not discriminate patients because of droopy eyelids or stiff expressions，and that they will assist the patients in returning to normal lives.

Jiang Xianping，from Hegang，Heilongjiang Province，45 years old，10 years with MG

Since I was living in a remote area，no one around me had any knowledge or understanding of MG. In addition to the physical pain caused by the illness，the lack of understanding of my family，relatives and friends made me even more sad. At that time，I really wanted to go to extremes. Fortunately，after treatment，I am now stable and content. Although there is a lot of financial pressure after the illness，I am no longer alone because of the love and support of my family. Most people around me also got to understand my condition and stopped looking at me strangely. Furthermore，I know how to cherish everything，cherish life，cherish the people and things around me，and cherish each day of life.

Shang Qiuli，from Hengshui，Hebei Province，48 years old，9.5 years with MG

MG patients are angels with broken wings. The state should include the commonly used drugs taken by patients with myasthenia gravis within its medical insurance coverage，and provide more medical benefits，so that they can fly in the sky and bloom in the firmament.

Ma Yadan，from Luoyang，Henan Province，44 years old，7 years with MG

There is a group called "Beijing Aili Myasthenia Gravis Rare Disease Care Center". People in this group have the same disease but live in different places. The patients have always accompanied each other，so I always have a belief that I can recover and return to normal life. Getting out took nearly four years，and I felt afraid，helpless，and very painful during that time. I feel very fortunate to be alive today. The unwavering love of my family is my most solid backing，and my own beliefs keep me moving forward. I participated in the training of patient care workers，and in accompanying patients，I felt the natural connection，trust and support among patients. Further，I learned that it is important to treat my illness correctly and to dispel the psychological shadow associated with it.

Sang Fengtao，from Zaozhuang，Shandong Province，61 years old，11 years with MG

Having been diagnosed withMG，I have felt as if my life has fallen into a black hole. There are more medicines than I can finish every day，and I visit the hospital on a monthly basis. For the last 11 years，I have been crawling in the dark，but I look forward to seeing the light ahead. I hope I no longer need to take medicines，or run to the hospital every month，and I could participate in social activities as normal people do.

Zhou Ailing，from Yantai，Shandong Province，59 years old，over 5 years with MG

I am a patient with generalized MG. I had a tracheostomy in 2018，I was unable to be weaned off the ventilator for a long period of time. My family purchased a home ventilator at their own expense due to the high cost of the ICU. After being taken care of by my family，I slowly recovered，and then I could live without the ventilator. I have slowly recovered with the assistance of my family over the past few years. While I am unable to work，I am able to dress and eat on my own. My families are all very happy for that. I hope that more caring people will pay attention to Aili and focus on the difficulties of patients suffering from MG.

Fu Chengwei，from Fushun，Liaoning Province，42 years old，19 years with MG

Life is a practice，and illness is like the trials and tribulations of this practice. As we strive to overcome the disease, we will accumulate experience that will benefit us for the rest of our lives. By experiencing more bumps in the road, you will experience less difficulties in the future, and your life will become smoother. Do not complain about the aggression, but face it bravely, there will always be partners together to achieve our goals.

Jin Yinzhen, from Shantou, Guangdong Province, 39 years old, 22 years with MG

There are too many obsessions in one's life. Only by reconciling with the past, reconciling with yourself, and reconciling with the disease can you really start a new life!

Patient Story

Liu Hongbing，48 years old，from Linzhou，Henan Province. He was diagnosed with MG when he was a teenager，and once got a temporary recovery. After having relapse in 2008，he has been working hard，along with his family，to fight the sufferings caused by the disease. His wife bought him one bottle of milk and two boiled eggs every day，while she and the children were reluctant to have any. Not only is myasthenia gravis afflicting him，but also the entire family.

What is Myasthenia Gravis

Myasthenia gravis（MG）is an autoimmune disease caused by conduction disorders at the nerve-muscle interface. It is characterized by fluctuating degrees of fatigue，which may be more severe at night than in the morning and may improve with rest. It is not genetically inherited，not contagious，and may develop in anyone at any age in life.

MG can cause weakness and easy fatigue in any skeletal muscle throughout the body，leading to a range of muscle-related symptoms of weakness such as double vision，drooping eyelids，difficulty lifting the arms or legs，stiff and weak facial muscles，neck weakness，difficulty swallowing，unclear speech，and breathing difficulties. These symptoms can affect the patient's daily life in any situation，as if carrying a heavy load on their back.

MG is a neuroimmune disease that is often confused by many with other diseases that have similar symptoms，such as "amyotrophic lateral sclerosis（ALS）". However，severe muscle weakness can be caused by many diseases and is not necessarily evidence of MG. MG is a treatable and reversible condition，and compared to progressive diseases with similar symptoms，it is more like a snowflake.

According to the Chinese guidelines for the diagnosis and treatment of MG（2020 edition），it is estimated that there are more than 9，500 new cases of MG in China each year. The gender ratio of MG is generally believed to be 1∶1.8 between males and females. Three peaks of incidence occur among young women aged 18—45，children under 14 years old，and elderlies aged 70—74.

The social problems faced by young and middle-aged patients with MG are particularly severe. Other than health issues, they face higher economic burdens, difficulties in caregiving and a range of survival and ethical issues in employment, marital relationships, social integration and familial responsibilities.

As a chronic disease, MG patients experience strong fluctuations and are prone to relapse. The course of the disease lasts a lifetime and is associated with a high risk of disability and death. Long-term disease management is necessary, and the side effects of medication can cause various complications. Many "cautionary drugs" can cause disease fluctuations or exacerbations, making it difficult to treat even common illnesses.

Therefore, new drugs that are effective, associated with minimal side effects and are convenient to use will effectively improve the survival conditions of patients. At the same time, accessibility and affordability of drugs are important considerations that affect standardized treatment.

Currently, there are still many areas in China where MG is not covered by outpatient medical insurance, and medication cannot be reimbursed or the reimbursement ratio is too low. Many families, especially adult patients, cannot afford long-term treatment, resulting in high family debts, inability to receive standardized treatment, or even an abandonment of treatment. Some patients cannot bear the dual torment of economic pressure and disease, leading them to a path of despair.

Beijing Aili Myasthenia Gravis Rare Disease Care Center

Beijing Aili Myasthenia Gravis Rare Disease Care Center（Aili Care Center）was founded by Myasthenia Gravis patients in 2013. It is a nonprofit social organization that provide services to patients with Myasthenia Gravis holistically. As a 5A-level organization assessed by the Beijing Municipal Civil Affairs Bureau，Aili Care Center has provided since its inception direct services to over 13000 patients and their families.

With the commitment to “same destiny，same feeling，” Aili Care Center is dedicated to helping patients improve treatment compliance and self-rehabilitation management capabilities，reduce the risk of crisis and disability，achieve peaceful coexistence with disease and return to normal social life. Services that have been made available include consultation and accompaniment，rehabilitative education，medical assistance，social integration，community empowerment，and field advocacy. Projects carried out include the 6.15 Myasthenia Gravis Care Day，the China Myasthenia Gravis Conference，the Aili Rehabilitation Camp，the Aili Rehabilitation Kit，“Aili Warm Heart”，Yunlu College，the Community Care Worker System，“Help Powerless Women Live Strongly” etc. By the end of 2022，there have been a total of 400＋ patient rehabilitation and empowerment activities，6 large-scale industry conferences，4 large-scale patient surveys，300＋ medical staff collaborations，400＋ media reports，2000＋ self-media reports，9，000＋ donations to support patients，helping patients identify or purchase medication for 6，500＋ times，distributed 70，000＋ copies of rehabilitation manuals，and averaged 100,000＋ consultations and replies annually.

Vision：Every patient lives in peace with the disease

Mission：Promote social awareness of myasthenia gravis，promote the progress of patient groups and society，improve patients’ medical treatment and living conditions，promote doctor-patient communication and medical research，and help patients return to normal social life.

Objectives：popularization of disease treatment，normalization of rehabilitation of patients，normalization of life and work，and popularization of prevention and treatment.

Slogan：The power of love，changes you and me!

Chinese Red Cross Foundation

Founded in 1994, the Chinese Red Cross Foundation (CRCF) is a national public offering foundation with independent legal person status initiated and managed by the Red Cross Society of China and registered by the Ministry of Civil Affairs. With the mission of "protecting life and health, the Red Cross is by your side", the CRCF is committed to becoming "the most actionable, innovative and influential foundation in China", with the purpose of promoting humanity, the Red Cross spirit of fraternity and devotion is committed to improving the living and development conditions of human beings, protecting human life and health, and promoting world peace and social progress.

In 2008, 2013 and 2018, CRCF was awarded the title of "Grade 5A Foundation" in the national social organization assessment. In 2010, 2015 and 2021, it was awarded the title of "National Advanced Social Organization" by the Ministry of Civil Affairs consecutively. It has won the China Charity Award seven times with 8 public welfare programs, including: Red Cross Angel Project, Chinese RED Action, Smile Angel Foundation, Spring Rain Action, Little Angel Fund, "Rescue the Heart with a Packet of Medicine", "New Station · New Health" Fraternity Health Hospitals (Clinics), and ByteDance Humanitarian Relief and Aid Fund for Health Workers. CRCF was awarded the title of "National Model Unit of Disaster Relief" by the Central Committee of the CPC, the State Council and the Central Military Commission, and the titles of " Advanced Unit in the Development of Model Organizations", " Advanced

Primary CPC Organization" and "May 4th Red Flag Youth League Branch" among Central and State Organs，etc. CRCF is the first national foundation to pass ISO 9001 quality management system certification and the SGS NGO benchmarking review，and won the honors of "RCSC Drive to Fight COVID-19：Advanced Collective"，"Annual Transparent Foundation"，"Self-Discipline Transparency Award"，"Annual Model Foundation"，the first place in the "Public Influence List of Public Fundraising Foundations" and many other honors.

6.15 重症肌无力关爱日
MYASTHENLA GRAVIS DAY

爱的力量
改变你我

发起方：中国红十字基金会
Sponsor: Chinese Red Cross Foundation

出品方：北京爱力重症肌无力罕见病关爱中心
Manufacturer: Beijing Aili Myasthenia Gravis
Rare Disease Care Center

微信公众号

抖音

新浪微博