降低血友病患者致残率，构建罕见病全生命周期医疗保障体系，促进人口高质量发展

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摘要

《中共中央关于制定国民经济和社会发展第十五个五年规划的建议》提出在“十五五”期间实施健康优先发展战略。在加快健康中国建设、促进人口高质量发展两大战略决策指引下，未来五年中国的医疗服务和保障体系将持续转向以人民健康为中心，致力于为全民提供公平可及、系统连续、优质高效的健康服务和保障[1]。

在中国推进系统性改革提升全民健康水平的进程中，罕见病患者是一个不可忽视的弱势群体，罕见病防治也成为一个极具挑战的公共卫生问题。中国非常重视罕见病的防治工作，2016 年已将完善罕见病用药保障政策纳入《“健康中国 2030”规划纲要》。2025 年世界卫生组织通过题为《罕见病：全球卫生公平和包容的优先事项》的决议，敦促会员国“争取到 2030 年实现和扩大全民健康覆盖，确保包括罕见病患者在内的所有人在其生命全程中的健康生活和福祉”。中国参与推动了这项决议，体现了中国为包括罕见病患者在内的全人群提供健康服务和保障的信心和决心。

近年来全球罕见病领域药物研发加速，随着创新疗法的问世和应用，部分罕见病的治疗理念已发生根本性转变，治疗模式也演变为和慢性病相同的全生命周期健康管理模式，患者有希望通过长期规范化的治疗，享有和健康人一样的生活质量。中国在慢性病防控领域取得了世界瞩目的成就，并将发

① 本报告仅代表企业相关研究观点，不代表论坛主办单位和承办单位立场和观点。

展慢性病防治康管全链条服务，而以血友病为代表的部分罕见病，既具有和慢性病相同的典型特点，也已具备发展诊治康管全链条服务的基础条件。

中国一直高度重视血友病患者的诊疗和保障，目前已建立全国血友病登记系统，初步建成了全国血友病三级诊疗体系，并将儿童临时和规律替代治疗药物，以及成人临时替代治疗药物纳入了基本医保。但目前中国血友病临床治疗模式仍以“临时替代治疗”为主，成人患者的年出血次数高达 52 次[2]，30 岁以上患者残疾比例接近 90%[3]，18 岁以上成人患者就业率为 35%，结婚率仅为 16%[4]。

降低全年龄段血友病患者致残率，对提升人口健康水平和劳动力质量，减轻长期医疗和社会负担具有积极影响，对实现全民健康目标，促进人口高质量发展也具有重要意义。中国可以借鉴在慢性病管理领域积累的成功经验，以血友病为切入点，建立罕见病诊治康管全链条服务的范式，也为世界罕见病防治事业输出中国方案。

“十五五”期间，辉瑞愿意与医疗机构、慈善组织和行业伙伴携手，共同推进“血友病健康 2030”项目，积极支持中国探索建立符合本国国情的血友病诊治康管全链条服务模式，为血友病患者提供覆盖全生命周期的疾病管理和医疗保障支持。在本报告中，我们提出了三点建议供决策机构参考：

首先，制定明确的血友病中长期防治目标，在全国血友病登记系统现有数据分析的基础上，争取到 2030 年首先实现成人患者规律替代治疗比例的显著提升，同时成人患者致残率有所下降，到 2035 年实现全年龄段患者致残率的显著下降。

其次，提升创新疗法可及性，优化成人规律替代治疗的医保政策。对于能够将年治疗费用控制在基本医保基金可负担范围内的创新药物，按国家医

保药品谈判程序纳入医保药品目录，推动成人患者转变治疗模式，降低致残率。

最后，加强血友病中心能力建设，推进血友病质控指标体系建设，提升血友病诊断、治疗、康复、患者管理服务的质量同质化，逐步实现中国血友病临床治疗模式从“救急治残”向“全生命周期健康管理”的转变。

一、引言

中国在“十四五”期间建成了世界上规模最大的医疗服务[5]和保障体系[6]，《中共中央关于制定国民经济和社会发展第十五个五年规划的建议》提出，在“十五五”期间实施健康优先发展战略。国家卫健委则提出了到 2030 年居民人均预期寿命达到 80 岁，主要健康指标进入高收入国家行列的新目标[7]。在加快健康中国建设、促进人口高质量发展两大战略决策指引下，未来五年中国的医疗服务和保障体系将持续转向以健康为中心，致力于为全民提供公平可及、系统连续、优质高效的健康服务和保障[8]。

在中国推进系统性改革提升全民健康水平的进程中，罕见病患者是不能忽视的一个弱势群体，罕见病防治也成为一个极具挑战的公共卫生问题。中国已确认的罕见病超过 1400 种[9]，罕见病患者数量已突破 2000 万[10]。中国非常重视罕见病的防治工作，2016 年已将完善罕见病用药保障政策纳入《“健康中国 2030”规划纲要》，至今已发布两批国家罕见病目录，共收录 207 种罕见病，初步建立了包含登记、诊疗、转诊等功能的全国罕见病诊疗协作网，并将约 100 种罕见病药物纳入国家医保药品目录[11]。2025 年，中国还参与推动了世界卫生组织的决议《罕见病：全球卫生公平和包容的优先事项》，该决议敦促会员国“争取到 2030 年实现和扩大全民健康覆盖，确保包括罕见病患者在内的所有人在其生命全程中的健康生活和福祉”。这体现了中国为包

括罕见病患者在内的全人群提供健康服务和保障，到 2030 年基本实现健康公平的信心和决心。

近年来全球罕见病领域药物研发加速，随着创新疗法的问世和应用，部分罕见病的治疗理念已发生根本性转变，治疗模式也演变为和慢性病相同的全生命周期健康管理模式，患者有希望通过长期规范化的治疗，享有和健康人一样的生活质量。中国在慢性病防控领域取得了世界瞩目的成就，并将发展慢性病防治康管全链条服务，而以血友病为代表的部分罕见病，既具有和慢性病相同的典型特点，也已具备发展诊治康管全链条服务的基础条件，有机会借鉴慢性病管理经验，探索建立中国的罕见病诊治康管全链条服务模式，提升全生命周期健康服务和保障水平，降低疾病发展和致残死亡风险。

本报告将以血友病为例，分析现阶段中国罕见病防治现状和主要挑战，分享国际经验，并就“十五五”期间可以达成的目标和建议采取的政策措施提出建议，希望能够为中国推进罕见病医疗服务和保障体系建设提供有益参考，并为实现罕见病患者享有健康生活的长期愿景贡献微薄力量。

二、中国血友病防治现状及挑战

1. 中国已具备发展血友病诊治康管全链条服务的基础条件

血友病是一种隐性遗传性出血性疾病，是罕见病的代表性疾病之一，2018 年即被纳入中国《第一批罕见病目录》。血友病最常见的症状是出血，出血部位主要为关节和肌肉，如发生在颅内可直接危及生命。血友病还可能伴随长期的慢性疼痛，而关节腔出血会产生剧烈疼痛，导致患者无法正常上学或工作，生活质量很低。血友病常在儿童期起病，长期反复的关节、肌肉出血会造成关节功能障碍甚至残疾[12]。血友病需要终身用药，如不能坚持规范化治

疗，病程持续进展将严重影响患者身体机能，并增加医疗、康复、照护支出给患者及其家庭造成较大经济负担，具有和慢性病相同的典型特点。

自 20 世纪 80 年代以来，中国一直高度重视血友病患者的治疗和管理。历经 40 年的发展，中国血友病防治策略日益完善，防治体系建设也取得了突飞猛进的进展，不仅建立了全国血友病登记系统，还初步建成了全国血友病三级诊疗体系，并参考欧洲和亚太标准制订了中国血友病中心建设标准。

根据中国血友病制定的《血友病中心建设标准（2024 年版）》，按多学科管理职能将血友病中心分为三级：治疗中心、诊疗中心和综合管理中心。血友病治疗中心需要承担患者登记、随访、常规治疗（预防出血和急性出血治疗）和健康教育的职能；血友病诊疗中心需要具备明确诊断血友病，评估肌肉、关节功能，治疗相关并发症，以及进行物理治疗与康复的能力；血友病综合管理中心则需要具备完整的血友病专业和多学科综合管理能力，并为血友病诊疗中心和治疗中心提供培训和专业指导[13]。

2020 年，中国血友病协作组和中国罕见病联盟联合发起了血友病中心建设项目，截至 2025 年，共有 198 家中心完成现场审核和授牌，其中 18 家为综合管理中心，39 家为诊疗中心，141 家为治疗中心。全国血友病三级诊疗体系的建立，为发展血友病诊治康管全链条服务奠定了坚实基础。

2. 中国血友病临床治疗模式仍以临时替代治疗为主，导致致残率较高

血友病的常规治疗手段分为两种，一种是临时替代治疗，过去称“按需治疗”，即只在出血时给予治疗，这是一种传统的被动治疗手段。另一种是国内外临床指南均推荐的规律替代治疗，过去称“预防治疗”。因“预防治疗”在中文语境下容易产生歧义，让人误解为是“预防血友病的发生”，而非“预防出血”，因此由中华医学会血液学分会发布的《血友病治疗中国指南（2025 版）》已正式将“预防治疗”更名为“规律替代治疗”[14]。

血友病患者由于凝血因子缺乏导致凝血功能障碍，因此关节和肌肉会反复出血。规律替代治疗通过规律性输注凝血因子或者注射非因子制剂，能够保持患者正常的凝血功能，从而预防出血，保护关节，避免残疾，并使患者具备正常生活和劳动的能力，其治疗理念和糖尿病需要长期控制血糖，高血压需要长期控制血压等慢性病的治疗理念在本质上是一致的。

目前中国血友病临床治疗模式仍以“临时替代治疗”为主。研究显示，中国 18 岁以下血友病患者接受规律替代治疗的比例已达到 46%，但成人患者接受规律替代治疗的比例仅为 18%[15]，仍显著低于世界平均水平。在欧美等主要发达国家，成人血友病患者接受规律替代治疗的比例在 80%以上，而在哥伦比亚、马来西亚等部分发展中国家，成人血友病患者接受规律替代治疗的比例也超过了 90%[16]。

由于中国血友病患者主要采取“痛了才治、出血才补”的临时替代治疗模式，导致预后不佳，致残率居高不下。临时替代治疗虽然可以在短时间内止血、缓解疼痛，但不能阻止下一次出血的发生，而关节的每一次出血都可能带来不可逆的损伤，长期反复出血会导致关节功能受损甚至残疾。2021 年中国的一项真实世界研究结果显示，在以临时替代治疗为主的模式下，成人血友病患者的年出血次数高达 52 次[17]。2024 年的中国血友病患者调研结果则显示，13～17 岁患者的残疾率为 17.24%，18～30 岁患者为 62.58%，31～45 岁患者为 88.19%，45 岁以上患者为 87.10%[18]。

3. 血友病成人患者疾病负担较重，亟需进一步提升医疗保障水平

近年来国家医保局持续加大罕见病保障力度，自 2017 年起开始通过医保谈判的方式将罕见病药物纳入国家医保药品目录，有效提升了罕见病药物的保障水平。截至 2025 年底，国家医保药品目录中罕见病用药的累计数量达到 136 个，覆盖了第一批和第二批罕见病目录中的 69 个病种[19]。

血友病作为第一批被纳入罕见病目录，且致残率较高的病种，受到了各级医保部门的关注和政策支持。截至 2025 年 12 月，共有 7 种血友病治疗药物被纳入国家医保药品目录[20]，包括儿童临时和规律替代治疗药物，以及成人临时替代治疗药物。儿童规律替代治疗药物被纳入基本医保，有效改善了血友病患者在儿童期的预后效果，也减轻了患儿家庭的经济负担。

目前成人血友病患者只能报销临时替代治疗的药品费用，疾病负担仍较重。一方面，很多成人患者因无法承担规律替代治疗的费用而选择临时替代治疗；另一方面，由于临时替代治疗属于对症治疗模式，患者因反复出血、关节病变、手术治疗、并发症处理等导致的医疗支出和社会成本更高。2024 年对中国血友病患者的疾病负担研究[21]显示，由于接受规律替代治疗的比例偏低，73.48%的患者出现关节病变，29.65%的患者在一年内接受过手术治疗，其中主要是关节置换术，而单次血友病关节置换手术的费用高达 13.7 万元。患者年人均直接医疗费用超过 45 万元，年人均自付医疗费用超过 10 万元，年人均总间接费用超过 5 万元，其中主要是误工或失业给患者及家属造成的损失。

此外，血友病患者及其家属是焦虑症及抑郁症的多发人群[22]，成人患者的身体功能、社会功能和心理健康水平普遍比正常人低[23]。如果患者在儿童期通过规律替代治疗能保持正常生活和学习，在成年后由于无法负担持续治疗的费用，导致失业、残疾，甚至因病致贫、因病返贫，在精神上和心理上也将遭受更大的打击和创伤。考虑到最早接受规律替代治疗的一批儿童患者已经或即将成年，因此优化成人规律替代治疗的医保政策，不仅有助于巩固和延续患者儿童期的治疗成果，也将帮助患者在成年期维持正常肢体功能，释放其作为青壮年劳动力的生产力，为社会创造经济效益。

三、全球血友病治疗和管理模式的演变

1. 创新疗法在患者全生命周期均展现出显著优于传统规律替代治疗的临床结局

近年来非因子类药物、基因治疗等创新疗法的问世，代表了全球血友病药物创新的新趋势，也因其显著优于传统疗法的临床优势为全球血友病患者带来了新希望。非因子类药物作为新一代规律替代疗法产品，在药理机制和给药途径上都突破了传统凝血因子类规律替代治疗药物的局限性，在提高患者依从性的同时能够保障长期治疗目标的实现。

传统的规律替代治疗药物虽能提升体内凝血因子水平，但血药浓度的峰谷波动使得患者在低谷期仍面临亚临床出血风险。非因子类药物通过恢复凝血酶的生成能力，实现凝血功能正常化，从而在生理层面阻断出血事件，预防关节损伤的发生。

此外，传统的规律替代治疗药物通过静脉注射给药，对于需要终身用药的血友病患者而言，隔日或每周两至三次的输注频率，不仅容易导致外周静脉通路耗竭，进而引发治疗中断，也严重限制了患者工作、出行与社交的自由度。非因子类药物通过皮下注射给药，大幅降低了注射次数，且剂量固定，既避免了患者高频静脉注射的治疗负担，提高了患者依从性，也便于医保费用的测算、监测和管理。

2. 以实现健康生活为目标，全球血友病临床治疗正在向全生命周期健康管理模式转变

血友病是因基因突变导致的遗传性疾病，目前尚无法完全预防和根治，但患者可以通过主动采取规律替代治疗，减少出血发生，维持正常的关节和肌肉功能，达到功能性治愈的结果。因此，世界卫生组织（WHO）、世界血友

病联盟（WFH）、美国血友病基金会（NFH）在其指南中均推荐规律替代治疗作为最主要的血友病治疗方法。

随着上述非因子类药物等创新疗法的临床应用，全球血友病临床治疗的长期目标已发生重要变化，从为所有患者提供规律替代治疗升级为帮助所有患者实现零出血的目标[24]。2020 年，全球血友病治疗领域提出了“血友病正常化”的全新理念[25]，即通过长期、规范化的规律替代治疗和综合性疾病管理，最终让血友病患者在身体、心理和社会层面实现“正常化”，享有和健康人一样的生活质量。

“血友病正常化”理念正在推动全球血友病临床治疗向全生命周期健康管理模式转变，治疗目的不再满足于延长患者的预期寿命，而是在减少关节损伤和预防自发性出血的基础上，确保患者可以不受限制地进行正常家庭和社会活动，并能承受轻微创伤，甚至在遭受严重外伤或手术的情况下无需特殊干预手段就能够恢复健康[26]。

非因子类血友病创新治疗药物已在中国获批上市，《血友病治疗中国指南（2025 版）》也充分肯定了此类药物的革新优势，并推荐将非因子类药物用于规律替代治疗。通过遵循指南推荐的规范化治疗方案，合理应用非因子类药物，中国的临床医生也有望帮助更多血友病患者实现从“维持生存”到“健康生活”的跨越。

四、政策建议

中国血友病的患病率在 2.73 ~ 3.09/10 万，截至 2023 年 6 月，全国血友病登记系统中包括血友病在内的遗传性出血性疾病病例为 4 万余例[27]。降低全年龄段血友病患者致残率，对提升人口健康水平和劳动力质量，减轻长期

医疗和社会负担具有积极影响，对实现全民健康目标，促进人口高质量发展也具有重要意义。

“十四五”期间，中国的血友病医疗服务和保障体系取得了重大进展，血友病全国三级诊疗体系基本建成，儿童规律替代治疗药物已被纳入基本医保。“十五五”期间，中国可以探索建立符合本国国情的血友病诊治康管全链条服务模式，为血友病患者提供覆盖全生命周期的疾病管理和医疗保障支持，具体建议如下：

1. 制定明确的血友病防治目标，提升规律替代治疗比例

如前所述，目前中国开展血友病规律替代治疗（即“预防治疗”）的比例仍低于发达国家，且规律替代治疗主要集中在儿童患者群体。这种以临时替代治疗（即“按需治疗”）为主的治疗模式，客观上给血友病患者带来的高致残风险不容忽视。2026 年 3 月，由中国血友病协作组、中华医学会血液学分会血栓与止血学组、中国罕见病联盟联合发布的《血友病关节损伤与致残风险现状白皮书》显示，成人患者中 75%存在“靶关节”问题（即同一关节半年内出血超过 3 次），83%患有合并症，其中血友病性关节病以 83.9%的发生率位居成人合并症首位。另有研究显示，成人血友病患者的社会功能参与度显著偏低，18 岁以上成人患者就业率为 35%，结婚率仅为 16%[28]。

建议国家卫生行政主管部门根据在全国血友病登记系统现有数据分析的基础上，制定明确的中长期血友病防治目标，到 2030 年首先实现成人患者规律替代治疗比例的显著提升，同时成人患者致残率有所下降，到 2035 年实现全年龄段患者致残率的显著下降。各省级卫生行政部门可根据当地医疗资源布局、血友病患者分布、血友病病例数据分析等实际情况，制定具体工作计划，逐步提升本区域内血友病患者接受规律替代治疗的比例，并降低致残率。

2. 提升创新疗法可及性，优化成人规律替代治疗的医保政策

目前已纳入国家医保药品目录的血友病治疗药物有凝血因子类药物和人凝血酶原复合物。临床治疗使用最广泛的重组人凝血因子药物在治疗成人血友病患者时，只有用于临时替代治疗情况下（即出血后使用）才能享受基本医保报销待遇。考虑到血友病患者自费承担长期规律替代治疗的能力有限，优化成人规律替代治疗的医保政策将有助于提升创新疗法可及性，同时减轻患者经济负担，避免因病致残带来的巨大照护负担与社会成本。国内外药物经济学研究结果也表明，相较于临时替代治疗，规律替代治疗是具有显著经济学价值的优选方案。

建议医保部门从血友病患者临床实际需求出发，评估成人规律替代治疗创新药物的长期临床获益和社会经济效益，对于能够将年治疗费用控制在基本医保基金可负担范围内的创新药物，按国家医保药品谈判程序纳入医保药品目录，这将有助于已接受规律替代治疗的儿童患者在成年后能够坚持治疗，同时推动成人患者转变治疗模式，降低致残率，让更多患者回归正常工作和生活，也提升医保基金的使用效率和成效。

3. 加强血友病中心能力建设，提升医疗服务质量同质化

《血友病治疗中国指南（2025 版）》已正式确立了规律替代治疗作为血友病常规治疗的基石地位，并明确提出对于所有重型患者以及反复发生出血的中间型患者，应尽早进行规律替代治疗。对已有关节损伤的成人患者，也应尽早开始并长期维持三级规律替代治疗，以延缓关节损害进展。

为进一步规范规律替代治疗的临床实践，建议在卫生行政主管部门指导下，鼓励行业内企业和罕见病慈善组织，共同支持中国血友病协作组和中国罕见病联盟，继续推进血友病中心能力建设，推进血友病质控指标体系建设，提升血友病诊断、治疗、康复、患者管理服务的质量同质化，逐步实现中国血友病临床治疗模式从“救急治残”向“全生命周期健康管理”的转变。

首先，血友病治疗中心主要承担常规治疗工作，建议继续加大临床医生培训力度，普及非因子类创新疗法，促进规律替代治疗的规范化；其次，血友病诊疗中心和综合管理中心需要为患者制定全面的治疗和康复训练方案，建议继续加强其多学科能力建设，并以“出血次数减少”、“残疾率降低”作为患者治疗成果量化的主要指标；第三，为进一步提高各级血友病中心的患者管理能力，建议在完善患者信息管理系统的基础上优化分工和协作机制，定期开展患者教育，及时进行患者随访，指导患者积极主动预防血友病可能并发的骨骼肌肉功能障碍。

五、结语

全民健康是建设健康中国的根本目的[29]，罕见病作为全球卫生公平和包容的优先事项之一，是实现全民健康道路上需要解决的挑战和难题。为了不让任何一个罕见病患者掉队，中国正在构建“早发现、早诊断、早治疗、可管理、有药用、能负担”的罕见病全链条诊疗与保障模式。血友病是罕见病的代表性疾病之一，也是一种致残率较高的罕见病，发展规范化的血友病诊治康管全链条服务，不仅能够有效控制残疾的发生和发展，也将为完善罕见病全链条诊疗与保障模式积累宝贵经验。

中国血友病防治事业的发展凝聚了几代血液病学专家的心血和不懈努力，也离不开政府部门、医疗机构、行业企业、慈善组织和社会各界的大力支持。“十五五”期间，通过加快转变血友病临床治疗模式、优化规律替代治疗医疗保障政策、提升血友病诊疗服务质量同质化等举措，中国有望在血友病防治方面取得突破性进展，为数万血友病患者创造健康未来，并为其它罕见病的防控建立范式，为世界罕见病防治事业输出中国方案。

“十五五”规划建议提出加快健康中国建设，促进人口高质量发展，“辉瑞中国 2030 战略”的长期愿景与此高度契合。我们将坚持以科学驱动，以患者为中心，加速引进全球前沿的创新疗法，支持提升疾病诊断能力与治疗标准，并推动本土科学创新发展。在罕见病领域，我们将以“行业协作者”和“生态共建者”的身份，推动罕见病医疗服务和保障体系的高质量发展，并与医疗机构、慈善组织和行业伙伴携手，共同推进“血友病健康 2030”项目，为中国血友病患者提供连续、规范、可负担的疾病管理与健康支持，帮助更多血友病患者实现“零出血、无残疾”的健康生活目标。

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Reducing the Disability Rate of Hemophilia Patients and Advancing High-quality Population Development by Establishing a Full-lifecycle Medical Security System for Rare Diseases

Pfize ①

Abstract

The *Outline of the 15th Five-Year Plan for National Economic and Social Development of the People's Republic of China* call for implementing a health-first development strategy during the 15th Five-Year Plan period. Guided by the two major strategic priorities of accelerating the "Healthy China" initiative and advancing high-quality population development, China's healthcare service and security system will continue to undergo a shift toward a people-centered approach over the next five years. This transformation aims to provide equitable, accessible, systematic, continuous, high-quality, and efficient health services and security for all citizens.[1]

In China's ongoing efforts to advance systemic reforms and enhance nationwide health outcomes, individuals with rare diseases represent one of the most vulnerable groups that cannot be overlooked. The prevention and treatment of rare diseases has become a public health challenge. China places great significance on the prevention and treatment of rare diseases, having incorporated the improvement of rare disease drug security policies into the *"Healthy China 2030" Blueprint* as early as 2016. In 2025, the World Health Organization adopted a resolution titled "Rare Diseases: a Global Health Priority for Equity and Inclusion," urging member states to "accelerate efforts toward achieving and extending universal health coverage by

① The views expressed in this report are those of the enterprise research and do not represent the official stance or opinions of the forum host and organiser.

2030, ensuring healthy lives and well-being for all individuals, including persons living with a rare disease, throughout their life course." China's active participation in advancing this resolution demonstrates its confidence and commitment to providing healthcare services and security for all people, including those with rare diseases.

Globally, drug development in the rare disease field has accelerated in recent years. With the emergence and application of innovative therapies, treatment approaches for certain rare diseases have undergone fundamental shifts. Treatment models have also evolved into comprehensive, lifelong health management approaches resembling those for chronic diseases. Patients now have the opportunity to achieve the same quality of life as healthy individuals through long-term, standardized treatment. China has made significant progress in the field of chronic disease prevention and control and is advancing the entire chain of services spanning diagnosis, treatment, rehabilitation, and health management for chronic conditions. The characteristics of certain rare diseases, such as hemophilia, parallel those of chronic diseases in several respects. The necessary conditions are also in place for the development of an integrated service model for hemophilia covering the entire chain of diagnosis, treatment, rehabilitation, and health management.

China has consistently prioritized the diagnosis, treatment, and healthcare security of hemophilia patients, establishing a national hemophilia registry system and developed a preliminary three-tiered national hemophilia treatment system. On-demand (episodic) and regular replacement therapy (prophylaxis) medications for children, as well as on-demand replacement therapy medications for adults, have been included in the Basic Medical Insurance (BMI) scheme. However, the current clinical treatment model for hemophilia in China remains predominantly on-demand replacement therapy. Adult patients experience an average of 52 bleeding episodes per year,[2] with nearly 90% of those over 30 years old developing disabilities. Further, 35% of adult patients over 18 years of age are employed,[3] while only 16% are married.[4]

Reducing the disability rate among hemophilia patients across all age groups has positive implications for enhancing population health and the quality of the labor force, alleviating long-term medical and social burdens, and achieving universal health coverage while promoting high-quality population development. China can leverage its successful experience in chronic disease management and use hemophilia as a focal point to establish a full-chain service model for the diagnosis, treatment, rehabilitation, and health management of rare diseases. This approach will also contribute Chinese solutions to global efforts in preventing and treating rare diseases.

During the 15th Five-Year Plan period, Pfizer stands ready to collaborate with healthcare institutions, charities, and industry partners to jointly advance the "Hemophilia Healthy 2030" initiative and actively support China in exploring a full-chain service model for the diagnosis, treatment, and health management of hemophilia tailored to its own circumstances. This model aims to provide hemophilia patients with disease management and insurance coverage throughout their entire lives. In this report, we offer three recommendations for policymakers to consider:

First, establish clear medium- and long-term prevention and treatment goals for hemophilia. Based on analysis of existing data from the National Hemophilia Registry, by 2030, the proportion of adult patients receiving regular prophylaxis can be increased, while disability rates among adult patients can be reduced. By 2035, the disability rate of patients of all ages can be significantly reduced.

Second, improve access to innovative therapies and enhance medical insurance coverage of prophylaxis for adults. Innovative drugs with annual treatment costs within the affordable range of BMI funds may be included in the National Reimbursement Drug List (NRDL) through the national negotiation process. This would facilitate a shift in treatment models for adult patients and thus reduce disability rates.

Finally, strengthen the capacity of hemophilia centers and develop a quality control indicator system for hemophilia. This would standardize diagnosis, treatment,

rehabilitation, and patient management services, gradually shifting the focus of China's clinical treatment model for hemophilia from emergency care and disability management to lifelong health management.

I. Introduction

During its 14th Five-Year Plan period, China established the world's largest healthcare service[5] and security system.[6] The *Outline of the 15th Five-Year Plan for National Economic and Social Development of the People's Republic of China* propose adopting a "health-first" development strategy during the 15th Five-Year Plan period. China's National Health Commission is setting new goals, aiming to raise the country's average life expectancy to 80 years and align its key health indicators with those of high-income countries by 2030.[7] Guided by two national strategic directives, accelerating the establishment of a Healthy China and promoting high-quality population development, China's healthcare system is set to continue to shift toward a health-centered model over the next five years. This transition seeks to deliver equitable, accessible, systematic, sustainable, high-quality, and efficient healthcare services and protection for all citizens.[8]

In China's ongoing efforts to improve the health of its population through systemic reforms, individuals with rare diseases represent one of the most vulnerable groups that cannot be overlooked. The prevention and treatment of rare diseases has become a public health challenge. To date, more than 1,400 rare diseases have been identified in China,[9] affecting an estimated 20 million people nationwide.[10] China attaches great importance to the prevention and treatment of rare diseases. In 2016, the *"Healthy China 2030" Blueprint* called for policies enhancing coverage for rare disease drugs. Since then, China has released two editions of its National Rare Disease Catalog, covering a total of 207 rare diseases. Furthermore, China has established a national collaborative network for the treatment of rare diseases, encompassing registration, diagnosis, treatment, and referral mechanisms. Approximately 100 rare disease therapies have also been added to the NRDL.[11] In 2025, China joined an initiative that led to the World Health Organization's adoption of a resolution on rare diseases, titled "Rare Diseases: a Global Health

Priority for Equity and Inclusion." This resolution urges member states to "accelerate efforts toward achieving and extending universal health coverage by 2030, ensuring healthy lives and well-being for all individuals, including persons living with a rare disease, throughout their life course." China's participation in this initiative demonstrates its confidence and determination to provide healthcare services and security for all, including rare disease patients, as part of its mission to essentially realize health equity by 2030.

Drug development in the field of rare diseases has seen rapid progress around the world in recent years. With the advancement and application of innovative therapies, treatment concepts for certain rare diseases have undergone a fundamental transformation. Treatment models increasingly resemble those for chronic diseases, with a focus on lifelong, continuous care. Through standardized, long-term treatment, many patients with rare diseases can now enjoy a quality of life comparable to that of healthy individuals. China has made great achievements in chronic disease prevention and control and intends to develop the full chain of related services, from prevention and treatment to rehabilitation and health management. The characteristics for certain rare diseases, such as hemophilia, parallel those of chronic diseases in several respects. The necessary conditions are also in place for the development of an integrated service model for hemophilia covering the entire chain of diagnosis, treatment, rehabilitation, and health management.

Leveraging China's experience with chronic disease management, hemophilia could serve as an example to explore a full-lifecycle care approach for rare diseases, enhancing the quality of healthcare services and security while reducing the risks of disease progression, disability, and mortality.

This report uses hemophilia as a case study to examine the current state and key challenges in the prevention and treatment of rare diseases in China. It also draws on international best practices to propose tangible goals and policy recommendations for the 15th Five-Year Plan period. We hope the report can inform China's ongoing efforts to strengthen its healthcare service and security system for

rare diseases, and contribute to the long-term vision of enabling patients with rare diseases to live healthy and fulfilling lives.

II. Current Status and Challenges of Hemophilia Prevention and Treatment in China

1. China Has Built the Foundation for a Full-Chain Service System for the Diagnosis, Treatment, Rehabilitation, and Health Management of Hemophilia

Hemophilia is a recessive inherited bleeding disorder and one of the most widely known rare diseases. Hemophilia was included in the first edition of China's *National Rare Disease Catalog*, released in 2018. The most common clinical manifestation of the disease is bleeding, most frequently occurring within the joints and muscles. If bleeding occurs in the skull, it can pose a life-threatening risk to hemophilia patients. Many hemophilia patients experience chronic pain. In particular, bleeding into the joint cavity can result in severe pain. As a result, patients may struggle to attend school or maintain employment, leading to a low quality of life. Typically emerging in childhood, hemophilia is characterized by recurrent episodes of bleeding into joints and muscles, leading to progressive joint dysfunction and, potentially, disability.[12] Patients require lifelong treatment, as the absence of sustained adherence to standardized therapy accelerates disease progression. This steady decline in physical function significantly increases the costs of medical care, rehabilitation, and caregiving, placing a considerable economic burden on patients and their families. In this sense, hemophilia shares many characteristics with chronic diseases.

Since the 1980s, China has always attached great importance to the treatment and health management of hemophilia. Over the past four decades, the country has steadily enhanced its national strategy for the prevention and treatment of hemophilia, achieving rapid progress in systemic development. China has established a national hemophilia registry, preliminarily developed a framework for

a three-tiered hemophilia care network, and introduced standards for national care centers based on European and Asia-Pacific standards.

According to the *Standard for the Establishment of Hemophilia Center (2024 Edition)* issued by the Hemophilia Treatment Center Collaborative Network of China, hemophilia centers are divided into three tiers based on their multidisciplinary management functions: treatment centers, diagnosis and treatment centers, and comprehensive management centers. Hemophilia treatment centers are responsible for patient registration, follow-up care, regular treatments such as routine prophylaxis and acute bleeding management, and health education. Diagnosis and treatment centers, on the other hand, must be able to provide definitive diagnoses, assess muscle and joint function, treat related complications, and provide physical therapy and rehabilitation. Comprehensive management centers are equipped with specialized capabilities and multidisciplinary management functions. These centers may also provide training and professional guidance to treatment centers and diagnosis and treatment centers.[13]

In 2020, the Hemophilia Treatment Center Collaborative Network of China and the China Alliance for Rare Diseases jointly launched a hemophilia center construction initiative. By 2025, 198 centers had successfully completed on-site evaluations and received accreditation. Of these sites, 18 were designated as comprehensive management centers, 39 as diagnosis and treatment centers, and 141 as treatment centers. The establishment of this national three-tiered system has built a strong foundation for the development of a full-chain service model encompassing hemophilia diagnosis, treatment, rehabilitation, and health management.

2. China's Clinical Treatment Model for Hemophilia Still Relies Primarily on On-Demand Replacement Therapy, Resulting in a High Disability Rate

Common clinical treatments for hemophilia fall into two categories. The first is episodic therapy, previously called "on-demand treatment," which is a traditional and passive approach that provides treatment only when bleeding occurs. The second is routine prophylaxis, also known as "regular replacement therapy" and previously known as "preventive treatment," which is recommended by domestic

and international clinical guidelines. The term "preventive treatment" can be misinterpreted in Chinese as preventing the occurrence of hemophilia rather than preventing bleeding. Thus, the *Chinese Guidelines on the Treatment of Hemophilia (2025)*, issued by the Thrombosis and Hemostasis Group of the Chinese Society of Hematology, Chinese Medical Association, officially renamed this approach "regular replacement therapy," or routine prophylaxis.[14]

Hemophilia patients experience coagulation dysfunction due to deficiencies in specific coagulation factors, resulting in recurrent bleeding in joints and muscles. Routine prophylaxis helps maintain normal coagulation function by regularly administering coagulation factors or non-factor therapies. This approach effectively prevents bleeding, protects joints, and reduces the risk of disability to enable patients to lead normal social and work lives. Fundamentally, this treatment concept mirrors chronic disease management models, such as long-term blood glucose control in diabetes or long-term blood pressure control in hypertension.

China's current clinical treatment model for hemophilia still relies mainly on on-demand replacement therapy. Research shows that while 46% of hemophilia patients under 18 years old in China receive routine prophylaxis, only 18% of adult Chinese patients receive the treatment, significantly lower than the world average.[15] In economically developed regions such as Europe and the United States, more than 80% of adult patients receive routine prophylaxis. In some developing countries such as Colombia and Malaysia, the proportion of adults receiving routine prophylaxis also exceeds 90%.[16]

Most hemophilia patients in China primarily receive on-demand replacement therapy, which addresses bleeding only after pain occurs and replaces coagulation factors only after bleeding has started. Thus, clinical outcomes remain poor, and disability rates remain high. While on-demand therapy can temporarily stop bleeding and alleviate pain, it cannot prevent future bleeding episodes. As each incident of joint bleeding can cause irreversible damage, recurrent bleeding over time usually leads to joint dysfunction and potentially even disability. A real-world study conducted in China in 2021 revealed that adult hemophilia patients

experienced 52 bleeding episodes annually when adhering to a treatment model predominantly based on on-demand replacement therapy.[17] Furthermore, survey results from 2024 indicate that the disability rate among Chinese hemophilia patients 13 to 17, 18 to 30, 31 to 45, and above 45 years of age was 17.24%, 62.58%, 88.19%, and 87.1%, respectively.[18]

3. Adult Hemophilia Patients Face a Large Disease Burden and Urgently Need Improvement in Healthcare Security

In recent years, the National Healthcare Security Administration (NHSA) has made significant strides in enhancing healthcare security for rare diseases. Since 2017, China has progressively added rare disease drugs to the NRDL through price negotiation mechanisms, greatly improving access to rare disease treatments. By the end of 2025, a total of 136 rare disease drugs were included in the NRDL, covering 69 diseases listed in the first and second editions of the National Rare Disease Catalog.[19]

Given its inclusion in the first edition of the National Rare Disease Catalog and high disability rate, hemophilia has garnered significant attention and policy support from healthcare security authorities at various levels. As of December 2025, a total of seven hemophilia treatments had been included in the NRDL.[20] These include drugs for both on-demand and routine prophylaxis in children, and on-demand replacement therapy in adults. The inclusion of routine prophylaxis drugs for children in China's BMI scheme has led to substantial improvements in childhood clinical outcomes, while also reducing the financial burden on families.

Under current policies, adult hemophilia patients in China are only eligible to receive reimbursement for medications used in on-demand replacement therapy, leaving them with a substantial disease burden. Many adult patients opt for on-demand treatment because they cannot afford the cost of routine prophylaxis. However, since on-demand replacement therapy only addresses symptoms rather than preventing bleeding, patients face higher medical and social costs due to recurrent bleeding, joint disorders, surgical interventions, and complication management. A 2024 study on the disease burden of hemophilia patients in China

revealed that, due to the low adoption of routine prophylaxis, 73.48% of surveyed patients had developed joint lesions and 29.65% underwent surgery within the past year.[21] The most common surgical procedure was joint replacement, costing RMB 137,000 (~USD 19,820) per case for hemophilia patients. These medical needs drove the average annual direct medical cost per patient to over RMB 450,000 (~USD 65,090), including an average annual out-of-pocket medical cost exceeding RMB 100,000 (~USD 14,460). In addition, the average annual indirect cost surpassed RMB 50,000 (~USD 7,230), primarily stemming from income loss due to missed work or unemployment affecting both patients and their families.

Moreover, anxiety and depression are highly prevalent among hemophilia patients and their family members.[22] Adult patients often experience lower levels of physical functioning, social engagement, and mental health compared to the general population.[23] This psychological and emotional burden can be even greater for those who, after benefiting from routine prophylaxis reimbursed by health insurance during childhood and leading a normal life with access to education, are unable to continue such treatment in adulthood due to its prohibitive cost. This transition can lead to unemployment, disability, or even poverty. Given the timeline of nationwide childhood treatment reimbursement, the earliest cohort of children who received routine prophylaxis has now entered or is nearing adulthood. Therefore, improving the health coverage of routine prophylaxis for adult patients will consolidate the treatment gains this generation achieved in childhood while helping adult patients maintain normal physical functioning. This, in turn, would allow hemophilia patients to remain active members of the workforce, generating economic value for society.

III. Evolution of the Global Hemophilia Treatment and Health Management Model

1. Innovative Therapies Demonstrate Optimal Clinical Outcomes Throughout Patients' Lifecycles Compared to Traditional Regular Replacement Therapy

In recent years, the emergence of innovative advancements such as non-factor products and gene therapy has marked a transformative shift in global hemophilia treatment. Owing to their significant clinical advantages over traditional therapies, these innovations have brought new hope to hemophilia patients worldwide. As a new generation of routine prophylactic treatments, non-factor therapies address the limitations of traditional factor-based treatment in pharmacological mechanisms and administration routes. They improve patient adherence while ensuring the realization of long-term treatment goals.

Traditional routine prophylactic treatments work by increasing coagulation factor levels. However, fluctuations in drug concentrations create "peak-and-trough" patterns, which can expose patients to subclinical bleeding during low-level episodes. In contrast, non-factor therapies restore thrombin generation capacity and normalize coagulation function, thereby preventing bleeding events at the physiological level and reducing the potential for joint damage.

In addition, traditional routine prophylaxis requires intravenous infusions. For patients that require lifelong medication, receiving infusions every other day or two to three times per week can exhaust peripheral venous access, leading to treatment interruptions. These frequent infusions also significantly limit patients' flexibility to work, travel, and participate in social activities. Non-factor therapies use subcutaneous injections, greatly reducing injection frequency, and adopt fixed dosing. These drug administration practices lower the treatment burden of frequent intravenous infusions, improve patient adherence, and standardize the calculation, monitoring, and management of healthcare insurance funds.

2. Aiming to Achieve Healthy Living, Global Hemophilia Treatment Is Shifting Toward a Full-Lifecycle Management Model

Hemophilia is a hereditary disease caused by genetic mutations, and while it cannot yet be fundamentally prevented or cured, patients can take proactive measures to manage the disease. Prophylaxis can significantly reduce the occurrence of bleeding and preserve normal joint and muscle function, achieving a functional cure. Therefore, the guidelines of the World Health Organization (WHO), the World

Federation of Hemophilia (WFH), and the National Bleeding Disorders Foundation (NBDF) recommend routine prophylaxis as the primary treatment approach.

The clinical application of innovative therapies, such as non-factor products, has significantly shifted the long-term goals of global hemophilia treatment. The focus has evolved from merely providing routine prophylaxis for all patients to striving for zero bleeding in every individual.[24] In 2020, the hemophilia field introduced a new concept called "normalization in hemophilia."[25] This concept emphasizes achieving normalization across physical, psychological, and social functioning through long-term, standardized prophylaxis and comprehensive disease management, allowing patients to enjoy a quality of life comparable to that of healthy individuals.

The concept of "normalization in hemophilia" is driving a shift in global clinical practice toward full-lifecycle health management. Treatment goals now extend beyond prolonging life expectancy. Instead, the focus is on minimizing joint damage and preventing spontaneous bleeding, ensuring that patients can participate in normal family and social activities without limitations. Furthermore, this approach aims to ensure that patients can tolerate minor injuries and, even in cases of severe trauma or surgery, recover without the need for extraordinary interventions.[26]

Non-factor innovative hemophilia treatments have already been approved in China. The *Chinese Guidelines on the Treatment of Hemophilia (2025)* have also fully acknowledged the advantages of these treatments and recommend their use in routine prophylaxis. By following standardized treatment pathways promoted by clinical guidelines and using non-factor therapies appropriately, clinicians in China are well positioned to help more patients transition from merely surviving to living healthy lives.

IV. Policy Recommendations

In China, the prevalence of hemophilia is estimated to be between 2.73 and 3.09 per 100,000 people. As of June 2023, more than 40,000 cases of hereditary bleeding disorders, including hemophilia, had been registered in the China National Hemophilia Registry. [27] Reducing the disability rate among hemophilia patients across all age groups is critical for improving overall population health and workforce quality. Moreover, it plays a positive role in mitigating long-term healthcare and social burdens. These efforts are essential for achieving universal health coverage and advancing high-quality population development.

During the 14th Five-Year Plan period, China achieved significant advancements in its hemophilia healthcare service and security systems. The country has preliminarily established a national three-tiered diagnosis and treatment network. Routine prophylaxis medicines for children have been included in the BMI reimbursement scheme. During the 15th Five-Year Plan period, China may consider exploring the development of a full-chain service model for hemophilia diagnosis, treatment, rehabilitation, and health management according to its national characteristics. Such a model would provide health management and healthcare security support for hemophilia patients throughout their lifecycle. Specific recommendations are as follows:

1. **Establish Specific Hemophilia Prevention and Treatment Targets and Increase Clinical Adoption of Routine Prophylaxis**

As previously mentioned, the proportion of hemophilia patients in China receiving regular replacement therapy (i.e., "prophylaxis") remains lower compared to developed countries. Pediatric patients constitute the primary population receiving prophylaxis. The prevailing treatment model, dominated by episodic replacement therapy (i.e., "on-demand treatment"), poses a high risk of disability for hemophilia patients that cannot be overlooked. In March 2026, the *White Paper on the Current Status of Joint Damage and Disability Risks in Hemophilia* was jointly released by Hemophilia Treatment Center Collaborative Network of China, Thrombosis and Hemostasis Group of Chinese Society of Hematology, Chinese Medical Association, and China Alliance for Rare Diseases. The report documented that 75%

of adult patients suffer from "target joint," defined as over three bleeding episodes within six months in the same joint, and 83% experience complications. Hemophilic arthropathy was identified as the most prevalent adult complication, affecting 83.9% of patients. Another study indicated that adult hemophilia patients exhibit markedly reduced levels of social participation. For adult hemophilia patients over 18 years of age, 35% are employed, while only 16% are married.[28]

It is recommended that national healthcare administrative authorities formulate clear medium- and long-term goals for hemophilia prevention and control based on existing data from the National Hemophilia Registry. By 2030, the proportion of adult patients receiving routine prophylaxis can be increased, while the disability rate among adult patients can be reduced. Further, a significant reduction in disability rates across all age groups can be achieved by 2035. Specific action plans may be developed based on local healthcare resource distribution, patient demographics, and case analysis. These plans may progressively increase the proportion of local patients receiving routine prophylaxis to reduce disability rates.

2. Improve Access to Innovative Therapies and Enhance Health Insurance Coverage for Adult Routine Prophylaxis

Currently, the NRDL includes hemophilia clotting factor drugs and human prothrombin complex concentrates. Among these are recombinant human clotting factor drugs, the most widely applied hemophilia treatment for adult patients clinically. However, under current NRDL terms, these drugs may only be reimbursed by BMI for adult patients for on-demand replacement therapies, namely those administered after bleeding episodes. Given the limited capacity of adult hemophilia patients to bear out-of-pocket costs for prophylaxis in the long term, expanding insurance coverage for adult routine prophylaxis would significantly improve access to innovative therapies. Expanding access to treatment would both reduce patients' financial burden and minimize disability-related caregiving demands and social costs. Pharmacoeconomic studies in China and abroad demonstrate that, compared with on-demand therapy, routine prophylaxis offers superior health economic value.

It is recommended that healthcare security departments comprehensively evaluate the long-term benefits and socioeconomic impact of innovative drugs for adult routine prophylaxis according to the clinical needs of hemophilia patients. Specifically, innovative drugs that have reasonable annual treatment costs and meet the affordability requirements of the BMI fund may be considered for inclusion in the NRDL through negotiation. This would help ensure that pediatric patients continue to receive routine prophylaxis during adulthood, while also enabling adult patients to transition to routine prophylaxis, thereby reducing disability rates. Moreover, this change would also help more patients return to normal work and daily life, while enhancing the efficiency and effectiveness of health insurance fund utilization.

3. Strengthen Capacity Building of Hemophilia Centers and Enhance the Standardization of Medical Services

The *Chinese Guidelines on the Treatment of Hemophilia (2025)* officially establish routine prophylaxis as the preferred primary treatment for hemophilia. The *Guidelines* recommend the early adoption of routine prophylaxis for all patients with severe hemophilia and for those with moderate hemophilia who experience recurrent bleeding. The *Guidelines* also recommend that adult patients with existing joint damage initiate and maintain long-term tertiary routine prophylaxis as early as possible to slow the progression of joint deterioration.

To further standardize clinical practice for prophylaxis, it is recommended that, under the guidance of health administrative authorities, various stakeholders work in cooperation to support the Hemophilia Treatment Center Collaborative Network of China and the China Alliance for Rare Diseases. These efforts would focus on building the capacity of hemophilia centers and advancing the development of a hemophilia quality control indicator system. Overall, these efforts would improve the consistency of diagnosis, treatment, rehabilitation, and health management services, gradually transitioning the clinical treatment model from addressing emergencies and disabilities to full-lifecycle health management.

First, it is recommended that hemophilia treatment centers primarily handle routine therapeutic tasks. Efforts may be further concentrated on training clinicians, promoting awareness of non-factor-based innovative therapies, and strengthening the standardization of prophylaxis. Second, it is recommended that hemophilia treatment centers and comprehensive management centers develop treatment and rehabilitation plans for patients and strengthen multidisciplinary treatment (MDT) capacity. To evaluate treatment outcomes, these institutions may consider adopting key quantitative indicators such as reduced bleeding episodes and lowered disability rates. Third, all levels of hemophilia centers can enhance their patient management capabilities by leveraging advanced information systems. These systems enable the centers to refine their functional roles and collaboration mechanisms, provide regular patient education, and follow up with patients in a timely manner. These measures will empower patients to prevent musculoskeletal dysfunctions associated with hemophilia.

V.Conclusion

Achieving universal health coverage constitutes the fundamental goal of building a "Healthy China."[29] Rare diseases, recognized globally as a priority for advancing health equity and inclusion, present unique challenges and complexities that must be addressed in the pursuit of universal health coverage. To ensure no rare disease patient is left behind, China seeks to establish a comprehensive rare disease diagnosis, treatment, and security model characterized by "early detection, early diagnosis, early treatment, health management, access to medication, and affordability." Hemophilia is a well-recognized rare disease that carries a comparatively high risk of disability. Establishing standardized, comprehensive services encompassing diagnosis, treatment, rehabilitation, and health management not only effectively mitigates the onset and progression of disability from hemophilia but also provides valuable insights for refining comprehensive models of rare disease care, covering diagnosis, treatment, and patient support.

The advancement of hemophilia prevention and treatment in China reflects the dedication and persistent efforts of generations of hematology experts. These achievements have benefited greatly from the strong support of government agencies, healthcare institutions, enterprises, charitable organizations, and all sectors of society. During the 15th Five-Year Plan period, China is well-positioned to make breakthroughs in hemophilia prevention and treatment by accelerating the transformation of treatment models, improving health coverage for routine prophylaxis, and enhancing the consistency of healthcare services. These efforts will create a healthier future for tens of thousands of hemophilia patients, establish an exemplary model for the prevention and treatment of other rare diseases, and enhance China's contribution to the global cause of rare disease management.

The *Recommendations of the Central Committee of the Communist Party of China for Formulating the 15th Five-Year Plan for National Economic and Social Development* call for accelerating the construction of a Healthy China and promoting high-quality population development. Pfizer's long-term vision under its China 2030 Strategy aligns closely with these strategic directives. Pfizer will remain committed to science-driven, patient-centered principles, accelerate the introduction of global cutting-edge therapies, support the enhancement of diagnostic and treatment standards, and advance domestic scientific innovation. In the field of rare diseases, we will continue to act as an industry collaborator and ecosystem partner, driving the high-quality development of healthcare service and security systems related to rare diseases. Working alongside healthcare institutions, charitable organizations, and industry partners, we aim to advance the "Hemophilia Health 2030" initiative and provide continuous, standardized, and affordable disease management and healthcare support for China's hemophilia patients, ensuring that more patients achieve the healthy life goals of zero bleeding and zero disability.

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