Biotechnology Innovation Organization

August 6, 2020

Center for Drug Evaluation
National Medical Products Administration
People's Republic of China
128 Jianguo Road, Chaoyang District, Beijing 100022
国家药品监督管理局药品审评中心
北京市朝阳区建国路 128 号，100022

RE: Technical Guideline on Clinical Trials of Cellular Immunotherapy Products (Exposure Draft)
关于公开征求《免疫细胞治疗产品临床试验技术指导原则（征求意见稿）》意见的通知

Dear Sir/Madam:

尊敬的先生/女士：

The Biotechnology Innovation Organization (BIO) appreciates the opportunity to submit comments in response to the *Technical Guideline on Clinical Trials of Cellular Immunotherapy Products* (Draft Technical Guidelines). We commend the China Center for Drug Evaluation, National Medical Products Administration (NMPA), for its efforts to address regulation around these important therapies.

生物技术创新协会 (BIO) 很高兴能有机会对《免疫细胞治疗产品临床试验技术指导原则》（征求意见稿）提出意见。我们赞赏中国国家药品监督管理局 (NMPA) 药品评审中心为解决有关这些重要疗法的法规问题所做的努力。

BIO is the world's largest biotechnology trade association representing biotechnology companies, academic institutions, and related organizations across the United States and in more than 30 other nations. BIO member companies are involved in the research and development of innovative biomedical, agricultural, industrial, and environmental biotechnology products.

BIO 是全世界最大的生物技术贸易协会，代表美国以及其他 30 多个国家/地区的生物技术公司、学术机构和相关组织。BIO 的成员公司主要研发创新型生物医学、农业、工业和环境生物技术产品。

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In the healthcare sphere many of BIO's member companies are involved in the research and development of cutting-edge transformative products and technologies including cellular therapies. These therapies have the potential to cure intractable diseases, bring hope to patients in need, and change the way we think of disease. However, it has only been in the past decade that research and development for cell therapies has brought the real potential of these products to light.

在医疗保健领域，BIO 的很多成员企业都参与研发前沿的变革性产品和技术，包括细胞疗法。这些疗法有望治愈顽固性疾病，为有需要的患者带来希望，并改变我们认知疾病的方式。然而，仅仅就是在过去十年里，细胞疗法的研发才实现了这些产品的真正潜力。

As regulation often lags behind scientific advancement, current regulatory frameworks directly addressing these products and technologies are sparse, and in some ways, nascent. Nevertheless, several countries have taken the initiative to develop guidelines in this area. BIO commends China for joining the growing number of countries preparing its regulatory system for the proliferation of these innovative therapies. BIO further recognizes China's efforts to provide more regulatory transparency as per the requirements of the Technical Barriers to Trade (TBT) Agreement of the World Trade Organization (WTO), as well as other relevant international transparency commitments, by allowing for a notice and comment period.

由于监管经常滞后于科技进步，直接涉及这些产品和技术的监管框架少之又少，甚至在某些方面仍处于初期阶段。但是，有些国家已开始制定该领域的指导原则。越来越多的国家已开始为这些创新性疗法的发展调整其监管体系，中国也是其中一员。BIO 对此表示赞赏。通过启动征求意见期，中国根据世界贸易组织 (WTO)《技术性贸易壁垒》(TBT) 协议的要求以及其他相关透明度的国际承诺提升了监管透明度，BIO 对此也表示肯定。

However, to achieve legitimate policy objectives such as the protection of human health and safety, BIO believes a more robust commenting period may be warranted. In the section pertaining to *Format and Guidelines for Notification Procedures for Draft Technical Regulations and Conformity Assessment Procedures* of the TBT Agreement, the WTO recommends "a normal time limit for comments on notifications of 60 days." Such a time period would allow for translation of the notice or guideline, and also provide for proper consideration of the important topics discussed. Topics such as clinical trial design, selection of patient population for clinical trials, dosing and treatment plans, and long term follow up are critical in any clinical development process; however, they can present specific challenges for cell therapies. Given that the global understanding of these types of therapies are still evolving, adequate time for public commenting is especially important. Furthermore, given the novel and developing applications of cell therapy products, and in the spirit of fostering a more efficient clinical development process, BIO recommends early and frequent communication between the regulatory authority and the product sponsor in developing clinical trials.

但是，为了实现正当的政策目标，如保护人类健康和安全，BIO 认为可能有必要延长征求意见期。在有关 TBT 协议的《技术法规草案和合格性评估程序通知程序的格式和指南》一节中，WTO 建议“征求意见期的正常时限为发出通知后的 60 天”。由于需要翻译通知或指导原则，以及妥善考虑相关重要问题，因此需要有上述时间。临床试验设计、临床试验的患者人群选择、给药和治疗方案以及长期随访等问题在任何临床开发过程中都至关重要；但对细胞疗法来说，这些问题又会带来特殊的挑战。鉴于全球各国对细胞疗法的了解仍在不断变化，给公开征求意见留出足够时间就尤为重要。此外，鉴于各种新型及不断发展的细胞疗法产品应用，本着促进更高效临床开发过程的精神，BIO 建议监管机构与产品申办方在开发临床试验方面尽早频繁沟通。

BIO's preliminary assessment of the Draft Technical Guidelines is that it provides important information to help companies navigate the cell therapy space in China. BIO was pleased to see references to relevant ICH guidelines throughout the document and we applaud NMPA's membership in the ICH as well as its position on the ICH Management Committee. BIO believes that NMPA's continued adherence to, and implementation of the three tiers of ICH guidelines, coupled with the requisite training, will serve to address many of the regulatory concerns associated with cellular therapies.

BIO 对《免疫细胞治疗产品临床试验技术指导原则》（征求意见稿）进行了初步评估，认为此草案提供了重要信息，有助于企业熟悉中国的细胞疗法领域。BIO 很高兴地看到在整个草案中均提到了相关国际协调理事会 (ICH) 指南，我们对药监局作为 ICH 的成员及贵局在 ICH 管理委员会中任职表示赞赏。BIO 认为药监局持续遵循和实施 ICH 指南的三个层面，并进行必要的培训，将有助于解决许多与细胞疗法相关的监管问题。

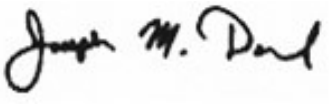
BIO's member companies are committed to bringing innovative cell therapies to the patients that so desperately need them. While BIO's comments are preliminary in nature, BIO looks forward to an opportunity to work with the NMPA to ensure that these cell therapies are safe, efficacious, and high quality, and meet appropriate regulatory requirements while at the same time allowing the timely access of these products to the patients.

BIO 的成员公司致力于为有迫切需求的患者带来创新型细胞疗法。尽管 BIO 的意见属于初步意见，但我们期待有机会能与药监局合作，确保这些细胞疗法安全、有效、高质量，并符合相关监管要求，同时能够让患者有机会及时获得这些产品。

BIO is grateful for the opportunity to submit comments for consideration by the Center for Drug Evaluation. Please contact Joseph Damond, at jdamond@bio.org, if have any question or if you would like to request additional consultations.

BIO 非常感谢能有机会提出意见，供药品评审中心考虑。如有任何疑问，或者您希望咨询其他问题，请通过 jdamond@bio.org 与 Joseph Damond 联系。

Sincerely,
谨致问候



Joseph M. Damond
乔戴盟
Executive Vice President for International Affairs
国际事务部执行副总裁

Addendum: Introduction to Biotechnology Innovation Organization (BIO)
附录：行业协会简介 - 生物技术创新协会（BIO）

Addendum
附录

Biotechnology Innovation Organization (BIO)
行业协会简介 - 生物技术创新协会（BIO）

The Biotechnology Innovation Organization (BIO) is the largest biotechnology not-for-profit trade association in the world, representing over 1,000 biotechnology enterprises, academic institutions, biotech R&D and innovation centers, and related organizations from more than 30 nations. In 2017, BIO joined the International Council for Harmonization for pharmaceuticals and serves on the ICH Management Committee. BIO's member companies are involved in the research and development of hundreds of innovative healthcare, agricultural, industrial and environmental biotechnology products, from cutting-edge regenerative medicines and medical diagnostics to renewable fuels and bio-based plastics.

美国国际生物技术创新协会 (BIO) 成立于 1993 年，是全球最大的生物技术贸易协会。BIO 代表总部位于美国及其他 30 多个国家/地区的 1,000 多家生物技术企业、学术机构、生物技术研发和创新中心以及相关组织。 BIO 在 2017 年加入了国际人用药品注册技术协调会（ICH），并成为 ICH 管理委员会成员。BIO 的公司成员参与了数以百计的创新医疗保健、农业、工业和环境生物技术产品的研究与开发，涵括从尖端再生药品和医疗诊断到可再生燃料和生物基塑料等领域。

BIO corporate members range from entrepreneurial companies developing a first product to Fortune 500 multinationals. The vast majority of BIO's member companies are small and medium sized enterprises (SMEs), including many innovative life-sciences start-ups, but BIO also represents multinational pharmaceutical companies that have a portfolio of biotherapeutic products. SMEs are a critical innovation force in the biomedical industry, responsible for 70% of the global clinical pipeline and 84% of all Orphan-designated programs.

BIO 的企业成员范围广泛，从正在开发第一个产品的初创公司到《财富杂志》500 强的跨国公司应有尽有。BIO 公司成员中绝大多数为中小型企业，其中包括许多创新型的生命科学初创企业，但 BIO 也代表了拥有系列生物治疗产品的跨国制药公司。中小型企业是生物医药业的关键创新力量，占据全球 70%的临床开发渠道及全球 84% 的罕见疾病的治疗指派计划。

BIO also works towards enriching the industry with networking, partnering and education opportunities. We organize the BIO International Convention, the global event for biotechnology, along with many other industry-leading investor and partnering events held around the world. Arguably the industry's leading partnering software, BIO **One-on-One Partnering™** system

facilitates 50,000 face-to-face meetings each year among investors, biotech companies, pharmaceutical companies, academic institutions and non-profits at live events.

BIO 组织了许多国际活动，包括每年的 BIO 国际生物技术展览大会、全球性的交流论坛以及在世界各地举行的投资者与合作伙伴关系会议活动。毋庸置疑，BIO 的合作伙伴关系项目- BIO One-on-One Partnering™ - 不仅在生物技术行业内同类最优，还每年在 BIO 展览现场促成了投资者、生物技术企业、制药公司、学术机构及非营利组织间 50,000 多次的面对面会谈。

Socio-Economic Trends and Industry Reports
社会经济趋势和行业报告

In addition to convening biotechnology leaders and experts and providing platforms for collaboration and exchange, BIO also conducts industry and trends analyses and sponsors research initiatives to formulate, evaluate, and promote policy solutions that accelerate innovation and promote economic growth and progress. BIO's 2020 Emerging Therapeutic Company Investment and Deal Trends Report, for example, highlights key investment and partnering activities in emerging therapeutic areas and provides industry, policymakers, and other stakeholders with a comprehensive view of trends driving global biomedicine development.

除了聚集生物技术领袖和专家以及为合作和交流提供平台外，BIO 还开展行业和趋势分析及赞助研究计划，以规划、评估和推进加速创新和促进经济增长与进步的政策解决方案。例如，BIO 的《2020 年新兴治疗公司投资和交易趋势报告》重点报告了新兴治疗领域的重要投资和合作伙伴关系活动，并为行业、决策者及其他利益相关方提供了有关驱动全球生物医药发展的趋势的综合观点。