

2025 ARRIGE Annual Meeting

IGBMC, Strasbourg (France) & Online



MEETING SUMMARY & STATEMENT

The international meeting brought together leading scientists, ethicists, clinicians, regulators, and industry representatives to address one of the most pressing challenges in contemporary biomedicine: the **global access to and affordability of gene therapies**. Opening remarks established the foundations for a truly multidisciplinary dialogue, spanning scientific innovation, regulatory frameworks, economic constraints, and ethical responsibility. The keynote framed the central question that guided the entire meeting: **can gene therapies realistically become accessible, affordable, and scalable worldwide?** A central message emerged clearly—accessibility will depend on the combined evolution of basic science, delivery technologies, intellectual property management, and innovative business models.

Discussions underscored that the **high cost of current gene therapies** is driven by multiple interconnected factors, including technological complexity, individualized manufacturing processes, regulatory burdens, and intellectual property frameworks. Several speakers emphasized the need to streamline regulatory processes, by focusing on platform technology approvals, and cautioned that some failures will necessarily occur and should not be allowed to halt or unduly slow continued efforts. The pricing of high-cost gene therapies must not ignore the voluntary and invaluable contributions of medical research participants by providing such therapies within programs which are based on reciprocity and sharing of the cost of therapy development

Advances in genome-editing platforms, delivery systems, and manufacturing technologies were identified as potential drivers of future scalability and cost reduction—if affordability is deliberately embedded early in the design of these technologies. Regional perspectives from Asia, Africa, Latin America, and Europe revealed profound **inequities in access**, shaped by disparities in health system capacity, reimbursement mechanisms, regulatory infrastructure, and technical resources. These discussions brought into sharp focus the unresolved ethical tensions between **innovation incentives and global equity**, particularly regarding public funding, pricing strategies, and corporate responsibility.

The final roundtable synthesized several converging priorities for action. These included strengthening international cooperation on governance and cost transparency, expanding equitable licensing models and public–private partnerships, and ensuring that the voices of low-resource settings are meaningfully integrated into upstream scientific, regulatory, and policy decision-making. The meeting concluded with a shared commitment to advance policy-relevant research, ethical guidance, and sustained international dialogue to promote fair access to gene therapies without undermining scientific innovation. Participants from both private industry and academia reaffirmed the shared responsibility of governments, companies, and the scientific community in shaping a more equitable future for advanced therapies.

At the same time, participants emphasized the importance of **recognizing existing efforts to facilitate technology adoption**, even though gene therapy technologies remain immature and will continue to evolve. In this context, the role of leadership emerged as critical—who these leaders are, the sectors in which they operate, the conditions that enable their effectiveness, and the environments required to allow more leaders to emerge. Equally important is the need to identify which efforts generate the greatest impact, recognizing that **public health priorities differ across regions and populations**, and that the most impactful solutions may not always involve genomics, even when genomics is the central focus of the debate.

A key structural tension was also highlighted between, on one hand, **genomic cures as high upfront investments with long-term returns**, which challenge health systems not designed for advance payment models, and, on the other, the collective burden of rare diseases, which individually affect small numbers of patients but together represent a major public health challenge. For pediatric rare diseases, the life-threatening nature of the conditions may justify faster access to therapies and more flexible regulatory pathways, whereas for diseases like sickle cell anemia the core challenge lies in providing basic treatments such as hydroxyurea and building the infrastructure required to deliver advanced therapies in Africa. Addressing this tension will require not only technological advances but also regulatory flexibility and new economic frameworks.

With the excitement generated by the unprecedented opportunities offered by advanced therapies comes a **profound responsibility**. Ensuring that all people, in all countries, can benefit from these innovations demands that a significant share of our collective effort be dedicated to upholding the **principle of equity** as a central pillar of scientific progress and global health.