



Gene therapy mini-series



DOLON

Support in the development of the report was provided by Dolon

Introduction

Realizing the potential of gene therapy: how do we pave the path to patient delivery and access?

The scientific community has achieved remarkable progress in developing innovative therapies, including gene therapies. While gene therapies offer the potentially groundbreaking opportunity to improve outcomes for individuals with rare and severe diseases, their adoption introduces distinct challenges for health systems that are currently tailored for more conventional medicines. As such, there is a significant opportunity to improve care delivery and increase patient access to current and future gene therapies.

In October 2022, Pfizer and the Alliance for Regenerative Medicine (ARM) organized a roundtable at the World Health Summit, assembling a mix of stakeholder representatives to explore advances in gene therapy, discuss existing health system barriers and deliberate on potential solutions. The roundtable focused on two specific areas where actions can be targeted: care delivery for gene therapy and patient access to gene therapy. Within these two areas, specific barriers and opportunities were identified:

Care delivery for Gene Therapy

| Barriers | Opportunities |
|---|---|
| Lack of infrastructure for diagnosis and determining patient eligibility for gene therapy | <ul style="list-style-type: none">• Support Multi-Stakeholder Education• Strengthen Infrastructure for Newborn Screening (NBS)• Develop Governance Frameworks for Eligibility and Referrals |
| Lack of infrastructure for gene therapy delivery | <ul style="list-style-type: none">• Certify National Centers of Excellence (COEs)• Integrate Hub-and-Spoke Model• Connect COEs across Countries |

Access to Gene Therapy

| Barriers | Opportunities |
|---|---|
| Challenges for the appropriate value assessment of gene therapies | <ul style="list-style-type: none">• Engage in Early Dialogue• Review Value Assessment Methodologies to Ensure Processes are Fit for Gene Therapies |
| Challenges to the adoption of innovative financing models | <ul style="list-style-type: none">• Support Horizon Scanning and Education• Support the Use of Novel Payment Models |

Following the success of the 2022 roundtable and recognizing the breadth of the topic, Pfizer, ARM, and roundtable participants saw a need to further the conversation and dedicate more time to exploring concrete actions for improving gene therapy delivery and access.

Pfizer and ARM subsequently convened a follow-on roundtable at the 2023 World Health Summit, gathering academics, scientific societies, healthcare professionals, patient advocates, payers, and policymakers from Europe, Latin America, Middle East, North America, and Oceania, with the aim to expand the discussion on the following topics:



Promoting multi-stakeholder education to support access to gene therapy



Enhancing the delivery of gene therapy through specific care models: examining centers of excellence



Facilitating effective health technology assessment for gene therapy



Novel payment models to facilitate patient access to gene therapy

Each paper in this four-part series will delve into the opportunities and best practices that emerged for these topics.