

Advancing Gene Therapy

AGT-2023

April 17-19, 2023 / Boston, MA

Preliminary Conference Agenda

April 17, 2023

Day-1

07:00-08:00 Registration and Welcome Speech by Conference Chairs

08:00-10:00 Session-1: Gene Therapy Pipeline: Targets

Disease Areas Amenable to Gene Therapy

Gene Therapy Targets

- Metabolic
- CNS
- Oncology
- Dermatology
- Neurology
- Ocular
- Others

10:00-10:45 Plenary Talk

Details will be updated soon

10:45-11:00 Refreshments Break

11:00-12:30 Session-2: Gene Therapy Pipeline: Challenges

Identify Challenges, Solution Pathways

Gene Therapy Research Challenges

- Medical Ethics in Gene Therapy
 - Equipoise (individual/institutional)
 - Vulnerable and Other Populations Requiring Additional Protections
- Local (organ specific/contained) vs Systemic
- Long term safety
- Persistence
- Cost
- ROA

12:30-13:30 Lunch and Networking Break

13:30-15:00 Session-3: Clinical Development: Preclinical/Nonclinical

Advances in Translational Medicine

Gene Therapies in Early Development

- Innovations in vectors
 - Vectorology
 - Non-viral and synthetic vectors
- Exploratory
 - Approaches to Gene Editing
 - Genetic engineering in organoids
- Preclinical/Non-clinical
 - Proof of Concept
 - Mechanism of Action

15:00-15:45 Plenary Talk

Details will be updated soon

15:45-16:00 Refreshments Break

16:00-18:00 Session-4: Clinical Development: Road to FIH

From Proof of Concept to Clinical Stage

- Phase I
 - Natural History
 - ROA
 - FIH strategies
 - Endpoint strategies
 - Recent updates: IFDD (FDA), PROs, COAs, ICH-E6/ICH-E9
 - Biomarkers, surrogate biomarkers, and the FDA
- Safety
 - Immunogenicity
 - Immunomodulation
 - Germline/Somatic integration

20:00 onwards Networking Dinner

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Day-2

07:00-08:00 Registrations

08:00-10:00 Session-5: Clinical Development: Advanced Stage

From Promise to Patient

Gene Therapies in Advanced Development

- Trial designs
- Completed Phase II
- Registrational (PII/PIII) Stage
- Current Phase III

10:00-10:45 Plenary Talk

Details will be updated soon

10:45-11:00 Refreshments Break

11:00-12:30 Session-6: Infrastructure

The Road from Bench to Bedside

Regulatory Affairs, Legal, IP

- In-licensing (Corporate and Academic)
- Global Regulatory Strategies
- Patents in key countries versus the cost of broad global filings
- Special locations (e.g., China, etc.) in a changing landscape

12:30-13:30 Lunch and Networking Break

13:30-15:00 Session-7: Manufacturing

The Human Gene Edited Product

Early/Late-stage pre-approval CMC

- In-house production vs Outsourcing
 - Will the CMO/CDMO own part of my IP
 - JV, costs of long-term brick-and-mortar
- Delivery Networks (stability, storage)

15:00-15:45 Plenary Talk

Details will be updated soon

15:45-16:00 Refreshments Break

16:00-18:00 Session-8: Post-Market Approval

Delivering on the Promise

Manufacturing

- Scale-up Considerations
- Life cycle management

Pharmacovigilance

- Persistence
- LTFU requirements (5/15 years)
 - Current and emerging trends

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Day-3

07:00-08:00 Registrations

08:00-10:00 Session-9: Commercialization and Marketing

Measuring the Value Proposition

Medical Affairs

- Patient Advocacy
- HEOR

10:00-10:45 Plenary Talk

Details will be updated soon

10:45-11:00 Refreshments Break

11:00-12:30 Session-10: Commercialization and Marketing

Global Outreach

Global Market Access

- Strategies and Commercialization
- HTA (UK, NICE), Insurance companies (US), CHMP (EU)

12:30-13:30 Lunch and Networking Break

13:30-15:00 Session-11: Human Gene Editing Ecosystem

Turning the Data Crank

Biotechnology Ecosystem

- Biotech (research based)
 - Out-licensing companies
- CROs
 - Clinical trials, SMOs
- CMO

15:00-15:45 Plenary Talk

Details will be updated soon

15:45-16:00 Refreshments Break

16:00-18:00 Session-12: Human Gene Editing Ecosystem

Hope and Promise Flourish

Clinical Stage Ecosystem

- Knowledge Management
- New Developments
- From Rare to Chronic

Make sure you are at the forefront of the gene therapy industry. No matter where your interest lies, we have content, networking, and potential partners for you.

Registrations are open now!

Book your seat here: <https://biogatesc.com/events/Gene-Therapy/>