



GENE THERAPY FORUM
MARCH 23-25, 2026 | BOSTON, USA



3RD INTERNATIONAL CONFERENCE ON

HUMAN **GENE THERAPY** CONFERENCE

MARCH
23-25, 2026

VENUE: HILTON BOSTON
BACK BAY BOSTON
MASSACHUSETTS, USA

SUPPORTED BY

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BOSTON

www.genetherapyconference.com

INSTRUCTIONS FOR SPEAKERS

1. **Invited Talks:** Invited speakers will be allotted 17 minutes to present their results, followed by a 3-minute discussion period.
2. **Oral Talks:** Oral speakers will be allotted 12 minutes to present their results, followed by a 3-minute discussion period.
3. Please do not exceed the allotted time slot.
4. Speakers should have their presentations saved on a USB memory stick.
5. It is suggested to email a copy of the presentations to us as back up.
6. Please prepare the presentation in PPT files, PDF is not recommended.
7. **Basic AV setup will be provided:** laser pointer, cordless mike, desktop mike, sound system.
8. Laptops equipped with Windows 10, Office 2010 Pro English (Word, Powerpoint, Excel) and Adobe Reader are provided.
9. If your presentation files contain movies, please make sure that they are well formatted and connected to the main files. You may check your slides during the breaks.
10. Projectors are equipped with standard VGA connection ports. Mac users should bring their own adapter cord.
11. Please re-check this program prior to the conference to confirm if any changes have been made to your session.
12. Conference volunteers will move the mic during Q&A. Audience with questions may raise hand to receive the mic.

INSTRUCTIONS FOR E-POSTER

1. Poster must be prepared in PowerPoint (Max 2 slides).
2. Use high-resolution images and readable fonts.
3. Each presenter will have 7 minutes to briefly explain their poster during the poster session, Followed by 3 minutes for Q&A.

CO-CHAIRS



MITRA TAVAKKOLI, MD, PHARM D

VP of clinical development and Head
of Drug Safety at Sangamo
Therapeutics, USA



ARUN SRIVASTAVA

Professor
University of Florida College of
Medicine



JEFF CHAFFIN

Market Access Strategy and Contracting
Consultant Miles Payer Strategy
USA



HILDEGUND ERTL

Professor
The Wistar Institute
USA



SOPHIE BLONDEL

Founder and CEO
Fox-Bio, France

DAY-1, 23 MARCH, 2026

09:00–09:45 Registrations

09:45–10:00 Welcoming Speech: Conference Co-chairs

Session I: Technology Innovations & Gene Delivery Platforms

Session Chairs: **Tristan Jervis**, Impact Shine Communications, UK

Mitra Tavakkoli, Sangamo Therapeutics, USA

10:00–10:20 Title: Expanding Gene Editing Beyond the Liver
Devyn Smith, Arbor Biotechnologies, USA

10:20–10:40 Title: Development of Capsid- and Genome-Modified NextGen AAV Vectors; Nuclease-Free Gene Editing With AAV-B19 Hybrid and Chimeric Vectors
Arun Srivastava, University of Florida College of Medicine, USA

10:40–11:00 Title: TBA (Online)
Zhenghong Gao, Uni-Pioneers Bio-Med, Inc, USA

11:00–11:30 **COFFEE BREAK**

11:30–11:50 Title: Gene Therapy for X-linked Retinitis Pigmentosa
Daniel C. Chung, Beacon Therapeutics, USA

11:50–12:10 Title: Technology Innovations
Jeremy Shelley, Form Bio, USA

12:10–12:30 Title: Gene Delivery Technologies
Molly McGlaughlin, Revolution Biomanufacturing Inc, USA

12:30–12:50 Title: New Technologies to Advance Manufacture, Delivery and Quality in AAV Gene Therapy
Daozhan Yu, AAVnerGene, USA

Session 2: Clinical Development, Trials & Therapeutic Applications

Session Chair: **Arun Srivastava**, University of Florida College of Medicine, USA

12:50–13:10 Title: Gene Editing Therapy Development: Platform Design and Manufacturing Readiness
Kok-Seong Lim, Independent Consultant, USA

13:10–14:10 **LUNCH BREAK**

14:10–14:30 Title: Isaralgagene Civaparvovec (ST-920) Shows Positive Mean Annualized eGFR Slope in Adults With Fabry Disease: Topline Results From the Registrational Phase 1/2 STAAR Gene Therapy Study and Long-Term Follow-Up Study
Mitra Tavakkoli, Sangamo Therapeutics, USA

14:30–14:50 Title: Targeting DNA Methyltransferase to Restore Vision After Traumatic Optic Nerve Injury
Wai Lydia Tai, MEEI, Harvard Medical School, USA

14:50–15:10 Title: Rare Diseases and Early Phase Units
Chris A. Learn, Parexel, USA
Anita Mardian, Parexel, USA

15:10–15:30 **Title:** Genetic Testing in Clinical Trials
Rachel Smith, Parexel, UK

15:30–16:00 **COFFEE BREAK**

PANEL DISCUSSIONS

Panel Chair: Rachel Smith, Parexel, UK

16:00–17:00 **Title:** Building Durable Patient Partnerships
Melissa Penn, Bayer Pharmaceuticals, USA
Daya Verma, Novartis, USA
Chris A. Learn, Parexel, USA
Tristan Jervis, Impact Shine Communications, UK
Rachel Smith, Parexel, UK

17:00–18:00 **Networking Cocktails**

DAY-2, 24 MARCH, 2026

Session 3: Regulatory Landscape, Quality & Compliance
Session Chair: Tristan Jervis, Impact Shine Communications, UK

10:00–10:20 **Title:** Scalable and Execution-Ready GT Manufacturing
Mihir Shukla, Biopharma Consulting Solutions, LLC, USA

10:20–10:40 **Title:** PAIs vs Surveillance Inspections
Deyaa Shaheen, Xfda Pharma Advisors Consultanting Services, USA

10:40–11:00 **Title:** Concurrent US and EU Approvals Roadmap
Rajiv Gangurde, Parexel, USA

11:00–11:30 **COFFEE BREAK**

11:30–11:50 **Title:** Proof of Concept to IND Readiness: How IND-Enabling Materials De-risk Regulatory and Funding Milestone
Emily Moran, Vector Biolabs, USA

11:50–12:10 **Title:** TBA (Online)
Nagendra Chemuturi, Eli Lilly and Company, USA

Session 4: Collaborations, Partnerships & Crisis Strategy

Session Chairs: **Emily Moran, Vector Biolabs, USA**

Anis H. Khimani, Antylia Scientific, USA

12:10–12:30 **Title:** Technology Innovations & Collaborations (Industry Scale-Up Focus)
Rachel Legmann, Repligen, USA

12:30–12:50 **Title:** Issues & Crisis Management
Tristan Jervis, Impact Shine Communications, UK

12:50–14:00 LUNCH BREAK

14:00–14:20 **Title:** TfR1 CapX: A Primate-Free, FDA-Aligned Pathway for IV Administered AAV for Brain-Wide Gene Delivery Across Neurological and Neurometabolic Disorders
Andrew Steinsapir, Deerfield Management, USA

14:20–14:40 **Title:** Overcoming Barriers to Cell and Gene Therapy Commercialization
Curt Kugel, ZS, USA

14:40–15:00 **Title:** Parent and Advocacy Perspective
Amanda Moore, Angelman Syndrome Foundation, USA

15:00–15:20 **Title:** Commercialization and Market Access (Online)
Jeff Chaffin, Miles Payer Strategy, USA

15:20–15:40 **Title:** Development of Capsid- and Genome-Modified NextGen AAV Vectors; Nuclease-Free Gene Editing With AAV-B19 Hybrid and Chimeric Vectors
Arun Srivastava, University of Florida College of Medicine, USA

15:40–16:10 COFFEE BREAK

16:10–16:50 PANEL DISCUSSIONS

Panel Chair: Patrick Starremans, Solid Biosciences Inc, USA

Title: Analytical QC and Potency Assurance Challenges
Patrick Starremans, Solid Biosciences Inc, USA
Nermin Ibreljic, Sarepta Therapeutics, USA
Rajiv Gangurde, Parexel, USA
Tristan Jervis, Impact Shine Communications, UK

16:50–17:10 E-POSTER PRESENTATIONS

GT01 **Title:** Manufacturing and Supply Chain Strategies
José Vázquez, c-LEcta GmbH, Germany

GT02 **Title:** TBA
Mehmet Karaca, Sarepta Therapeutics, USA

DAY-3, 25 MARCH, 2026

Session 5: Manufacturing, Supply Chain & Commercialization Strategy Session Chair: Molly S. McGlaughlin Revolution Biomanufacturing Inc, USA

- 10:00–10:20** **Title:** FDA Inspection & GMP Expectations
Alonza Cruse, Office of Pharmaceutical Quality Operations, USA
- 10:20–10:40** **Title:** Low-Cost AAV Viral Vectors Can be Manufactured Profitably: Engineered HEK-293 Cells and New Tools Combine to Yield Stable, High-Productivity Producer Cells
Larry Forman, CHO Plus, Inc, USA
- 10:40–11:00** **Title:** Building a Scalable, AI-Enabled AAV Manufacturing Platform: From Molecular Design to Commercial-Ready Production
Brian Tomkowicz, SK Pharmteco, USA
- 11:00–11:30** **COFFEE BREAK**
- 11:30–11:50** **Title:** Selective Removal of Early-Eluting Genome-Containing AAV Species with Elevated Residual DNA *via* Optimized Anion Exchange Chromatography
Nermin Ibreljic, Sarepta Therapeutics, USA
- 11:50–12:10** **Title:** A Modified CRISPR Approach Using XIST for the Treatment of Down Syndrome
Volney Sheen, Harvard Medical School, USA
- 12:10–12:30** **Title:** Frontiers of Gene Therapy: Trends and Opportunities from Payload through Scale Up
Anis H. Khimani, Antylia Scientific, USA

PANEL DISCUSSIONS

Panel Chair: Michael DiBiasio-White, ViroSpark BioConsulting, USA

- 12:30–13:10** **Title:** Manufacturing and Supply Chain Strategies
Michael DiBiasio-White, ViroSpark BioConsulting, USA
David Greenwald, Deerfield Management, USA
Larry Forman, CHO Plus, Inc, USA
Tristan Jervis, Impact Shine Communications, UK
Mihir Shukla, Biopharma Consulting Solutions, LLC, USA
Daozhan Yu, AAVnerGene, USA

- 13:10–13:30** **Closing and Cocktails**



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WE WISH TO SEE YOU AT HUMAN GENE THERAPY CONFERENCE-2026



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