PRISM

GENE FORUM
September 26-27, 2022 | Online

International HUMAN GENE THERAPY CONFERENCE

September 26-27, 2022

Online

https://genetherapyconference.com/

Key Participants

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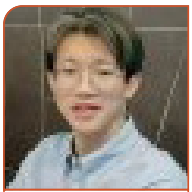

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Gene Forum 2022

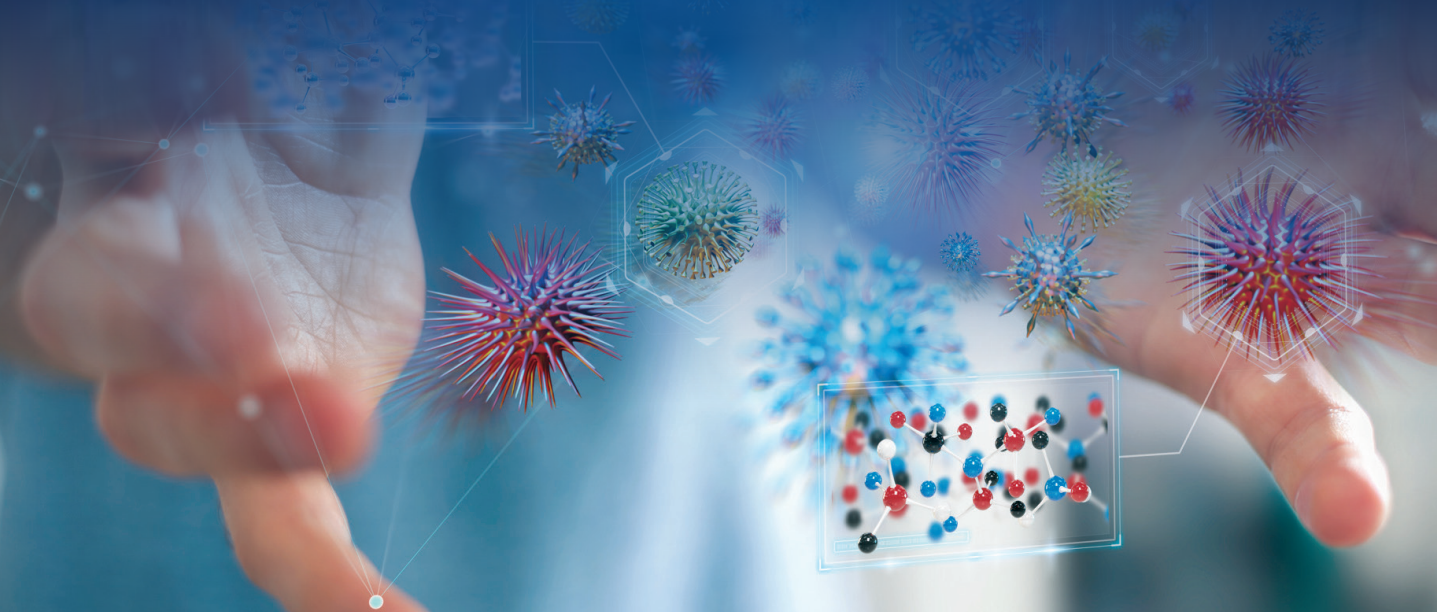
Gene Forum takes the honour to invite you to the **International Human Gene Therapy Conference** which will be held on **September 26-27** at **Online.**

Gene therapy, an experimental technique that uses genes to treat or prevent diseases, is experiencing a surge in interest and R&D investment. Over the past few years, gene therapy has become a regular talk for the scientists and researchers from academia and industry all over the globe. To date, over 2600 trials in 38 countries have been held. With the heightened interest and the push to move quickly from bench to clinic, it is imperative for companies to have a better understanding of the implications of regulatory, commercialization, preclinical and clinical strategies on their gene therapies.

Gene Forum 2022 will provide in-depth insights from 200+ industry's top scientists who will present the latest updates on how to improve efficiencies across all phases of Gene Therapy development and demonstrate the value of gene therapy products to regulators, payers, and patients.

It's a unique way to approach and encourage a dialogue between speakers and delegates through its well-planned agenda with the series of talks, poster presentations, panel discussions and networking events that will keep participants engaged in learning. This will help rising scientific queries and provide solutions for a smarter and more advanced future.

We are stepping forward to create a platform to bring all the researchers from around the world and the research community to collaborate with the industries to focus on their efforts. The industry partners and attending experts will also provide a fantastic networking experience.



Why to Attend?

- Find out the new technologies that uplift progress in this field
- Learn early industry best practices for gene therapy manufacturing from successful therapy launches.
- Understand the ethical considerations and challenges that surrounds gene therapy
- Get the latest update on vector design and development
- Build the most effective relationships with global and regional players and policy makers, to ensure patient access and commercial sustainability.
- Build strong and scalable delivery network, beyond the lab, that encourage multi-stakeholder collaboration.
- Deep analysis into successful strategies for post-launch manufacturing scale up and delivery network integration which will reduce your time and lower costs.
- Make your voice heard as a thought leader through interactive with 10+ sessions including live Q&A and panels.

Key Topics

- Global and Regional Case Studies
- Gene Therapy Raw Materials Customization
- Efficient Human Genome Editing
- Vector Systems and Manufacturing
- Building a Global Clinical Trial for Rare Disease Patients
- Frame Work and Ethical Practice for Gene Therapy in Healthcare System
- Navigating Gene Therapy Safety in Clinical Development
- Manufacturing and Commercializing Gene Therapies in Australia
- Selection and Management of Suppliers and Contract Service Provider
- Developing Translational Strategy to Set up for Preclinical & Clinical Programs
- Implication of Gene Therapy in Health Practice after Clinical Trial
- Next-Generation Sequencing Workflow in Future Evolution of Gene Therapy Development.

Learning Objectives

At the end of this conference, participants will be able to:

- Describe how this innovative therapy is transforming the biopharma industry and impacting people's lives.
- Interpret regulatory and quality requirements for gene therapy products with Australian regulatory insight.
- Explain how facility and equipment design can be optimized for the unique challenges of gene therapy.
- Identify innovative approaches to technology transfer that address the unique aspects of gene therapy manufacturing processes.
- Summarize the importance and considerations for product characterization and potency testing given the uncertainty inherent with the application of disruptive technologies inherent to gene therapy.
- Discuss the unique considerations for developing effective supply chain strategies to meet global requirements for gene therapy.

Who Will?

Department

- Manufacturing
- Product Development
- Quality
- Value & Market Access
- Therapy Administration
- Research & Development
- Regulatory Affairs
- Vector Systems
- Engineering
- Laboratory
- Science
- Information Technology
- Validation
- Training
- Clinical
- Patient Advocacy

Job Function

- Scientist
- Executive and Mid-Level Management
- Project Management
- Technical Services
- Supply Chain
- Manufacturing
- Application
- Risk Management
- Patient Monitoring
- Customer Engagement
- Marketing and Sales
- Clinical Strategy Pharmacist
- Product Communications
- Clinical Research
- Post Doc, PhD Student

Gene Therapy Market in Australia

Despite COVID-19, this trend has continued according to Melbourne-based Aus biotech, which reported in September that the global regenerative medicines sector attracted $19.9 billion of investment in 2020. Our goal extended to providing an overview of important sectors that impact the development of cell and gene therapy manufacturing in Australia and New Zealand.

In addition, there have been reports relevant to realizing the commercial and therapeutic potential of the regenerative medicine sector by the Australian Academy of Science, AusBiotech and the NSW Stem Cell Network, Stem Cells Australia, MTP Connect, the New Zealand MedTech Centre of Research Excellence (MedTech CoRE) and Consortium for Medical Device Technologies and the New Zealand Ministry of Science Research and Technology. The region also features in global industry and market reports.

Based on these estimates the following products were considered to be the most likely to enter the Australian market:

- Cell Therapy: StrataGraft® (Mallinckrodt Plc) and RVT-802 (Enzyvant Sciences Ltd) in 2023; Omidubicel (Gamida Cell Ltd) and Stapuldencel-T (Sotio AS) in 2024;
- Gene Therapy: Vutrisiran (Alnylam Pharmaceuticals Inc) in 2023; Fitusiran (Sanofi) and Etranacogene dezaparvovec (UniQure NV) in 2025; and
- Gene Modified Cell Therapy: Lisocabtagene maraleucel (Juno Therapeutics Inc) in 2023; Ciltacabtagene autoleucel (Legend Biotech Corp) in 2024.

Global Gene Therapy Market

The Gene Therapy market is segmented by Indication (Cancer, Metabolic Disorders, Eye Disorders, Cardiovascular Diseases, Spinal Muscular Atrophy and Others), Technology (Adeno Virus Vector, Adeno-associated Virus Vector, Lentiviral Vector, Retroviral Vector, Herpes Virus Vector and Others), and Geography (North America, Europe, Asia-Pacific, Middle East and Africa, and South America).

The global gene therapy market size was valued at USD 2.26 billion dollars in 2020, and is expected to grow at a compound annual growth rate of 28.32% from 2021 to 2026 to reach USD 11,739.75 million dollars.

Important factors that are driving the gene therapy market growth include recent approval of products such as Zolgensma and LentiGlobin. Spinal Muscular Atrophy (SMA) led the gene therapy market in 2020 with the highest revenue share of over 41%. However, the large B-cell lymphoma segment is estimated to account for the maximum revenue share by 2028.

Day 1

Monday, 26 September, 2022
Online

AEST	GMT	
09:30-09:45	23:30-23:45	Meeting (AV Check)
09:45-10:00	23:45-00:00	Opening Remarks and Introduction
10:00-10:30	00:00-00:30	**Extracellular vesicle delivered RNA directed transcriptional gene silencing** **Kevin Morris**, Griffith University, Australia
10:30-11:00	00:30-01:00	**First Gene Therapy Drug in Japan: Launch of Collategen** **Ryuichi Morishita**, Osaka University, Japan
11:00-11:20	01:00-01:20	Short Break
11:20-11:50	01:20-01:50	**It's Science, not fiction: the clinical application of genetic therapies in child neurology** **Michelle Farrar**, University of New South Wales, Australia
11:50-12:20	01:50-02:20	**Colon-targeted NAMPT-specific peptide systems for treatment of acute and chronic colitis** **Chul-Su Yang**, Hanyang University, Korea
12:20-12:50	02:20-02:50	**CRISPR gene editing via a light-triggered liposome system** **Wei Deng**, University of Technology Sydney, Australia
12:50-13:20	02:50-03:20	**Biophysical characterizations of AAV vectors for gene therapy** **Susumu Uchiyama**, Osaka University, Japan
13:20-13:40	03:20-03:40	Break
13:40-14:10	03:40-04:10	**Targeting choroidal vascular dysfunction via inhibition of circRNA-FoxO1 for prevention and management of myopic pathology** **Dan Li**, Fudan University, China
14:10-14:40	04:10-04:40	**Characterization of a Programmable Argonaute Nuclease from the Mesophilic Bacterium Rummeliibacillus suwonensis** **Xiaoman Jiang**
14:40-15:10	04:40-05:10	**Engineered Escherichia coli for biotechnological applications** **Vijai Singh**, Indrashil University, India

Day 2

Tuesday, 27 September, 2022
Online

AEST	GMT	
16:30-16:45	06:30-06:45	Meeting (AV Check)
16:45-17:00	06:45-07:00	Opening Remarks and Introduction
17:00-17:30	07:00-07:30	**Towards therapies for autosomal dominant Retinitis Pigmentosa using CRISPR/Cas9 gene editing** **Paul Thomas**, South Australian Genome Editing (SAGE) Facility, Australia
17:30-18:00	07:30-08:00	**MicroRNA replacement therapy for prostate cancer** **Jyotsna Batra**, Queensland University of Technology, Australia
18:00-18:30	08:00-08:30	**Onasemnogene abeparvovec in spinal muscular atrophy: an Australian experience of safety and efficacy** **Arlene D'Silva**, University of New South Wales, Australia
18:30-19:00	08:30-09:00	**An Australian clinical experience in treating children who have Spinal Muscular Atrophy with Zolgensma gene therapy!** **Sandra Charlton**, Sydney Children's Hospital Network, Australia
19:00-19:20	09:00-09:20	Short Break
19:20-19:50	09:20-09:50	**DNA and RNA Medicines: Chemical Evolution of Synthetic Delivery Carrier** **Ernst Wagner**, Ludwig-Maximilians-Universität(LMU) München, Germany
19:50-20:20	09:50-10:20	**Folate receptor-mediated delivery of Cas9 RNP for enhanced immune checkpoint disruption in cancer cells** **Ulrich Lächelt**, University of Vienna, Austria
20:20-20:50	10:20-10:50	**Decoy RNA-binding protein gene therapy of myotonic dystrophy** **Nicolas Sergeant**, University of Lille, France
20:50-21:20	10:50-11:20	**Evaluation and mitigation of off-target effects associated with CRISPR-Cas genome editing** **Julia Klermund**, University of Freiburg, Germany
21:20-21:40	11:20-11:40	Break
21:40-22:10	11:50-12:10	**Developing RNA-based gene targeting therapies against emerging respiratory viruses and viral cancers** **Adi Idris**, Menzies Health Institute Queensland, Australia
22:10-22:40	12:20-12:40	**A Second-Generation Nanoluc-IL27 Fusion Cytokine for Targeted-Gene-Therapy Applications** **Marxa Figueiredo**, Purdue University College of Veterinary Medicine, USA
22:40-23:10	12:50-13:10	**Challenges in establishing product reference standards for use with complex biologics** **Omar Dabbous**, Novartis, USA
23:10-23:40	13:10-13:40	**Development of NextGen, GenX, and Opt AAV Vectors for Human Gene Therapy** **Arun Srivastava**, University of Florida

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