Gene Therapies

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Gene therapy: What is it and how does it work?

Addressing the root cause of genetic disease

A new working copy of a missing/defective gene is isolated and placed inside a viral vector. The viral vector acts as an envelope to deliver the new gene to body's cells.

The vector is administered to a patient. It enters the cells, where it breaks down, allowing the new working gene to be introduced into the nucleus of the cells.

With the new gene in place, the body's cells begin producing the functional protein.

The gene therapy discovery journey

1989 Retrovirus vector
Gene transfer into cancer patients

1999 Adenovirus vector
Death of gene therapy patient (OTC deficiency)

2017 US FDA approval of first gene therapy
(for B-cell ALL; August)

Subretinal AAV2-mediated gene therapy for RPE65-associated Leber congenital amaurosis

2017 US FDA approval (December)

2020 Health Canada approval (October)

AAV9-mediated systemic gene therapy for SMA

2019 US FDA approval (May)

2020 Health Canada approval (December)

As of Q3 2023

27 gene replacement therapies approved in at least one jurisdiction

New heights of collaboration to deliver innovation

New approaches in working together: moving from bench to bedside to achieve regulatory approval, reimbursement and with healthcare systems to deliver to patients

Our SMA gene therapy:
Approved in >45 regions and countries

3,400+ patients treated globally
in our clinical trials, our managed access programs and in the commercial setting

All numbers are for continuing operations

A vital approach to reaching patients

We recognize the importance of collaboration with a range of stakeholders to successfully advance the care provided to patients.

MUSCULAR DYSTROPHY CANADA
DYSTROPHIE MUSCULAIRE CANADA

We have partnered with Muscular Dystrophy Canada to add spinal muscular atrophy to all existing provincial newborn screening panels.

The beginning of a new era

27 gene replacement therapies approved for clinical use*

Cancers – 14

- Head & neck cancer (2)
- Solid tumours (1)
- Melanoma (1)
- ALL / diffuse B-cell lymphoma / non-Hodgkin lymphoma / acute mantel cell lymphoma and/or follicular lymphoma (5)
- Multiple myeloma (3)
- Malignant glioma (1)
- Bladder cancer (1)

Neurometabolic – 7

- Adenosine deaminase deficiency (1)
- Leber congenital amaurosis (1)
- Spinal muscular atrophy (1)
- Metachromatic leukodystrophy (1)
- Early cerebral adrenoleukodystrophy (1)
- Aromatic L-amino acid decarboxylase (AADC deficiency (1)
- Duchenne muscular dystrophy (1)

Others – 6

- Critical limb ischemia (2)
- Transfusion-dependent □-thalassemia (1)
- Hemophilia A (1)
- Hemophilia B (1)
- Dystrophic epidermolysis bullosa (1)

*in at least one country / jurisdiction

https://asgct.org/global/documents/asgct-citeline-q3-2023-report.aspx

Disrupting for better outcomes

Gene therapies bring a new treatment platform and call for new ways of working together.

Thank you

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