An experimental gene therapy for DMD shows positive results in a three-patient trial

Cell & Gene Therapy Conference - London UK, October 2018

LONDON, UNITED KINGDOM, June 26, 2018 /EINPresswire.com/ -- SMi are delighted to welcome the expertise from a senior panel of big biotech's and pharma organisations, as well as regulatory bodies, at the inaugural Cell & Gene Therapy 2018 conference, taking place this October in the City of London, UK.

Did you know...
the global gene therapy market is forecasted to hit $363 million by 2022.

Latest News:

Positive Trial Results for Experimental DMD Gene Therapy

An experimental gene therapy for Duchenne Muscular Dystrophy (DMD) has showed better-than-expected results in a three-patient trial, according to preliminary data. Company shares jumped 60 percent following the news that the treatment dramatically boosted levels of microdystrophin, a muscle-protecting protein designed by researchers, and reduced levels of an enzyme associated with the disease.

DMD is a rare genetic disorder caused by loss-of-function mutations in the dystrophin gene. An X-linked condition, the disease mostly affects boys, and usually manifests itself in the form of muscle weakness in children between the ages of 3 and 5. There is no cure for DMD, and although steroids can slow the progression of symptoms, the disease eventually causes life-threatening damage to the heart muscles. Few patients live beyond their 30s. Part of the limitations, which have added friction to the commercialisation of therapies targeting neuromuscular disorders, are tied to the nature of the vectors used to deliver these treatments.

To hear more about this, join Giuseppe Ronzitti, Cell and Gene Therapy Specialist, Genethon as he will be presenting: 'Overcoming adeno-associated virus gene therapy limitations in genetic neuromuscular diseases.'

Presentation Details Below:

- Rare, genetic neuromuscular diseases constitute a bigger challenge given the absence of

knowledge on the physiopathological mechanisms and of therapeutic options

- Adeno-associated virus (AAV) vector-based gene therapy became a principal actor in the development of therapies for monogenic diseases

- Successful human trials of gene transfer in the liver for hemophilia A and B, in the eye for congenital blindness and in the nervous system for spinal muscular atrophy have unveiled the therapeutic potential of this viral vector platform

- Development of technological tools to overcome the current limitations of AAV gene therapy applied to neuromuscular diseases

Expert Speaker Panel:

Join an expert speaker panel at the Cell and Gene Therapy conference to get ahead and hear about latest developments! Presenting new technologies, specific case studies, updates on GMP, MHRA regulatory guidelines, quality control and therapeutic applications of stem cells, pre-clinical and clinical trial design, advanced cell engineering technologies, commercialisation of ATMPs, global pricing, ethical overviews and many more.

Hear from opinion leaders such as UCL, AstraZeneca, Selecta Biosciences, Plasticell, Innovate UK, LucidQuest + more.

- Discover the World of Advanced Therapy Medicinal Products -

For those looking to attend there is currently a £300 early-bird saving ending June 29th!

Further information is available at: www.cellandgeneconference.com/ein

SMi presents the Launch of:
Cell & Gene Therapy 2018
Date: 10th – 11th October 2018
Workshops: 9th October 21018
Location: Copthorne Tara Hotel, London UK
Website: www.cellandgeneconference.com/ein

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About SMi Group:

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