**CONFERENCE ON RARE DISEASE AND ORPHAN DRUGS**

Orphan drugs are prescription medicines designed for the detection, mitigation or treatment of life-threatening or crippling unusual disorders. They are 'orphans' since, under standard business circumstances, the pharmaceutical sector has no interest in producing and selling medications approved for such a limited group of people with extremely uncommon conditions. The critical public health problem is the increasing number of uncommon diseases which need care. The shortage of opportunities for drug makers and the absence of compelling data for the approvals also restrict the amount of potential experimental products that eventually enter the market.

Most of the uncommon disorders are triggered by single gene defects, sometimes arising as genetic illnesses with a clear sequence of Mendelian inheritance. Many of the medical breakthroughs in disease science over the past few decades have emerged from studies into genetic diseases. Clinical trials are carefully and ethically conducted in order to protect patients against unwanted adverse reactions and to allow collection and accurate analysis of the information concerning the disease. Pharmaceutical firms have been focused steadily on producing infectious disorder medicines. It is partially thanks to scientific advancements helping to recognize the molecular origins of infectious diseases.

The FDA Office for the Promotion of Orphan Products (OOPD) was established to recognize and support orphan goods. Rare diseases, as described broadly in the US Orphan Drug Act (ODA), are illnesses or disorders with an incidence of fewer than 200,000 individuals. Grants to Orphan drugs for clinical research are a validated way of effectively promoting and supporting the production of novel safe and efficient uncommon disease / condition medicinal goods. After 1983 the Orphan Products Grants Program has funded clinical testing trials and promoted the marketing acceptance of over 60 products. OOPD funded grants ensure that product development occurs in a timely manner with a very modest investment. To tackle the existing unmet demand and the shortage of care for certain uncommon diseases, the FDA is concentrating its attention on a potential funding mechanism to promote and push experimental drug treatments in healthy and effective ways by promoting creative clinical research approaches such as scalable and streamlined study formats, modelling and simulations, and basket and umbrella trials. A variety of legislative opportunities have prompted an increasing commitment by the biotech and pharmaceutical industry in the production. When this law was passed very few businesses were involved in producing orphan medicines. In reality, only about ten drugs for unusual diseases had been introduced into the market in the ten years before the Orphan Drugs Act was enacted. There has been a huge interest in the room for rare diseases, particularly by small biotech companies but also by bigger biopharmaceutical companies, according to the regulations. It culminated in well over 600 orphan medicinal products licensed to date.

**Why to Attend?**

Join the Conference on Rare Diseases and Orphan Drugs to stay up to date with the market and hear from our panel of speakers, bring you interesting new case reports, express their scientific opinions and comment on these specific topics.

* Gain knowledge of different uncommon diseases and their particular problems, including how to apply management approaches to certain rare diseases.
* Development of cell and gene therapies to cure genetic diseases-discover how gene therapy approaches can enhance severe disease care and why it is becoming more widely effective.
* Know how the legislative environment for product approvals is different across nations, and why staying updated about each region's legislation and guidance.
* This is a chance to connect with people across the world keen on finding out about Rare Diseases and Orphan Drugs.

**Key Highlights**

* One need to know about the recent development as well as strategies to tackle the issues associated with the disease.
* Regulatory Framework for Drugs for Rare Diseases
* To share the research experiences and bring awareness regarding these diseases to the outside world especially young researchers and student delegates
* The attendees can hear important presentations from leading suppliers of approaches, biotechnology firms, healthcare practitioners, health experts and community representatives exploring alternative strategies to promote the production of orphan drugs.

**WHO SHOULD ATTEND?**

* Physician
* Researchers
* Policy Makers
* Health Care Professionals
* Infectious Diseases Specialists
* Pharmacists
* Microbiologists
* Training Institutes
* Orphan Drugs Companies
* Rare Diseases Associations
* Parasitologists
* Epidemiologists
* Students

**Benefits of Attending the Conference**

* Provides an outstanding forum for researchers to discuss their success in Rare Diseases & Orphan Drugs relevant sessions spanning from fundamental science to treatment, biomarker-based disease planning and clinical development.
* It is a popular forum that puts together, on speech-provoking themes and ongoing work on infectious disease problems linked to emerging genetic disorder and orphan drug therapies.
* Share experiences, strategies and insight in interactive peer-to peer round tables and much more

**Speakers**

* **Jonathan Morton:** Agency lead, Comradis
* **Rich Horgan:** Founder & President, Cure Rare Disease
* **Lydia L. Seiders:** Patient Education Council Member, Aplastic Anemia & MDS International Foundation
* **Kelly Austin:** Founder, Regulatory Specialist (Odd, Fast Track, Breakthrough, RMAT)
* **Harsha K Rajasimha:** Founder and CEO, Jeeva Informatics Solutions
* **Alice Rathjen:** Co-Founder and CEO, DNA Compass