Bio^{M}-Seminar

Development of Cell & Gene Therapies: Risks and pitfalls and how to avoid them

Time and Place
Tuesday, 04.02.2020, 9:00 am –13:00 pm
Bio^{M} Biotech Cluster Development GmbH, Am Klopferspitz 19a (IZB West II, 3. OG), 82152 Martinsried

Speakers/ Abstracts

Dr. Kathrin Schneider, Sirion Biotech GmbH
Dr. Kathrin Schneider is VP Business Development and Sales at SIRION Biotech and a molecular biologist by training. Being with the company since early beginnings Kathrin was jointly responsible in refinement of the viral vector technology platform and building a global presence and client base. In her current position Kathrin is responsible for the global business strategy in supporting gene and cell therapeutic companies.

The successes seen in clinical studies on viral vector-based gene and cell therapies are well documented, with an ever-broadening pipeline of products entering late-phase clinical trials and more and more becoming licensed medicines. The majority of these therapies under development target devastating and potentially fatal diseases, which severely impact the quality of life of the patient and their families. For this reason, clinical development programs for innovative gene therapies have been actively accelerated by regulatory bodies such as the MHRA and the FDA by the application of a “Fast Track” or “Breakthrough” designation, enabling expedited access to these new medicines.
The flip side of accelerated pathways is that timelines for the manufacture of viral vectors become compressed, making it more difficult to develop commercial manufacturing processes that can produce vectors at the right quality, in the required amounts, and at costs that are reasonable enough to secure reimbursement from healthcare providers while still making financial sense to the developer. This talk addresses early stage possibilities in therapeutic viral vector development to ensure therapeutic efficiency, producibility and transfer into clinics in reasonable time.

Dr. Konstantin Petropoulos, Leukocare AG
Dr. Petropoulos is a biologist and chemist by training with focus on leukemia and stem cell research. Before joining Leukocare to head BD, Marketing & Sales, he was with Bayer AG and MorphoSys AG, serving several roles in science, business and commercial roles with increasing responsibility. He holds a PhD from LMU Munich and an MBA of FOM University of Applied Science.

In recent years approvals of both CAR-T cell therapies and promising clinical data of gene therapy trials such as in hemophilia or sickle cell disease, prove that significant progress of gene and cell therapy in the field of hematological malignancies as well as rare hematologic genetic disorders has been made. Replication-deficient recombinant viral vectors such as adenovirus serotype 5 (Ad5), adeno-associated virus (AAV) and lentivirus represent a rapidly growing field of vaccine development and gene therapy. Viral vectors are known as complex supra-molecular ensembles of macromolecules produced by living organisms which are prone to a variety of complex chemical and physical degradation pathways. Therefore, sufficient stability of gene and CAR-T products still represents a significant challenge and hurdle for the development and administration of stable vector-based pharmaceuticals such as vaccines or gene therapeutics. This talk will focus on current best practice and formulation weaknesses for gene and CAR-T therapies.

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Dr. Paula Salmikangas, NDA
Dr. Salmikangas is a member of the NDA Advisory Board and has background as a former Chair and member of the Committee for Advanced Therapies (EMA). She provides CMC, non-clinical and regulatory advice to companies developing ATMPs.

ATMPs include wide variety of cell / gene therapy and tissue engineering products, which have shown highly promising efficacy results. However, these products have very unique safety features and their manufacturing and quality control may be challenging, which have caused major hurdles for clinical translation and commercialization. This presentation will provide insight into:

- currently approved ATMPs and those in clinical development
- development challenges (CMC, pre-clinical and clinical) of ATMPs with few case examples
- key regulatory expectations for ATMPs

Agenda

09:00 am	**Registration**
09:10 am	**Opening welcome/Introduction** ***Dr. Andreas Berghammer, Bio^{M} Biotech Cluster Development***
09:15 am	**Viral vector to support gene and cell therapies from early stage – commercial engineer and manufacturing considerations** ***Dr. Kathrin Schneider, Sirion Biotech GmbH***
10:00 am	**Formulation Development for Gene Therapy: Do's, Don'ts & Clinical /Commercial Considerations** ***Dr. Konstantin Petropoulos, Leukocare AG***
10:45 am	**Refreshments & Networking**
11:15 am	**Translational challenges and regulatory expectations of ATMPs: CMC, pre-clinical and clinical aspects** ***Dr. Paula Salmikangas, NDA***
12:15 am	**Buffet & Networking**
13:00 pm	**End**

The seminar will be held in English.

Registration
Electronic registration under: http://www.bio-m.org/development-of-cell-and-gene-therapies
Registration fee: 80 Euro (+ VAT) for Academia, 130 Euro (+ VAT) for Industry

Organisation

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