

**LEUKOCARE's 7<sup>th</sup> Scientific Formulation Symposium**

On September 24, LEUKOCARE, yet again, welcomed a range of guests from academia and industry to celebrate the 7<sup>th</sup> Scientific Formulation Symposium at its facilities in Munich. This year's event was chaired by LEUKOCARE's new Chief Operating Officer, Dr. Andreas Seidl, who joined the management team in April 2019. Globally recognized speakers from academia, as well as from the biopharmaceutical industry, spoke about their current research and development work. This year's summit focused on three topics: **Formulation Development in the area of Viral Vectors, Biologics Product Development** and **Artificial Intelligence (AI)**. The exciting program led to lively discussions during the event and thereafter.

The first talk, "Formulation challenges of gene and cell therapy" by **Magdalena Obarzanek-Fojt of Novartis Pharma AG (Drug Product Leader for Cell and Gene Therapies)**, introduced the broad spectrum of ATMP development at Novartis. In her interesting talk, Magdalena focused on the differences between gene therapy and cell therapy products. She explained the challenges of formulation development in this area and showed that the combination of Design of Experiments (DoE) and the systemic screening of excipients allowed for the identification of lead formulations.

**Ian Pitfield, Vice President CMC of Gyroscope Therapeutics Ltd**, a gene therapy company based in the UK and US, held the second talk. Gyroscope's lead program employing an AAV-based vector in dry age-related macular degeneration (AMD) is currently in a Phase I/II clinical trial. Ian pointed out that current standards of formulation development for AAV gene therapies are not cost-effective for commercial supply to a broad patient population. Hence, new and innovative formulation approaches are required to support the commercial success of this class of game-changing medicinal products. Formulation development has been "under the radar" during the last few years, but is now gaining in importance due to the increasing number of programs of advanced therapy medicinal products and the challenges in providing them to a broader population.

The third talk "Formulation and Delivery Determine Effect – Formulations to Target the Immune System" by **Regina Scherließ, Director Dep. Of Pharmaceutics and Biopharmaceutics, University of Kiel**, highlighted the importance of new routes of administration for prophylactic vaccines. Regina impressively exposed why mucosal vaccines should be preferred over parenteral vaccinations. Mucosal vaccines allow for the activation of the different arms of the immune system and easy frequent boosting. In addition, mucosal administration is relatively easy, not requiring trained personnel.

Subsequently, **LEUKOCARE's Vice President BD, Marketing & Sales, Konstantin Petropoulos**, gave an overview of LEUKOCARE's R&D activities in the area of stabilizing viral vectors.



LEUKOCARE's 7<sup>th</sup> Scientific Formulation Symposium

Utilizing LEUKOCARE's customized data- and algorithm-based development approach in combination with a DoE-model successfully allowed for the stabilization of an adenovirus serotype 5 in both liquid and lyophilized formulations at 5 °C. Stabilization during lyophilization and storage for up to 18 months, as well as storage in liquid formulation for up to 24 months, could be reached in separate projects. Moreover, in a different non-disclosed, non-attenuated live enveloped virus, stabilization during lyophilization and storage for at least 12 months was achieved.

The second part of the formulation summit shed light on the pioneering, **algorithm-based formulation development** approaches, markedly new and innovative fields of activity in biopharmaceutical development, and a core expertise of LEUKOCARE.

**Johannes Buchner, Chair of Biotechnology, TUM Department of Chemistry, Technical University of Munich**, gave a thorough overview of antibody architecture and the formulation of protein folding solutions. In his talk, Johannes focused on the utilization of a genetic algorithm optimizing specific conditions (such as storage time) and yields in order to reduce the number of experiments compared to a multi-parameter space. The described screening strategy allows for recognizing and restricting limiting variables and is very much in line with LEUKOCARE's formulation development.

The ensuing talk by **Sabine Hauck, Vice President R&D at LEUKOCARE**, was about AI and Database Driven Formulation Development. AI widens the design space and enables the scientist to find new formulations by trying more combinations specifically tailored to the target product profile of the respective drug product. Furthermore, it reduces expensive and time-consuming lab efforts by shifting *in vitro* to *in silico*. As the amount of data is continuously increasing, the advantages of AI will become more prominent in the future.

As a culmination of the series of exciting talks, the audience enjoyed the presentation of **John Carpenter, Professor of Pharmaceutical Sciences and Co-Director of the Center for Pharmaceutical Biotechnology, University of Colorado**, who provided outstanding insights on interactions of stabilizing excipients with proteins in a WebEx broadcasted live from Denver. Being one of the most distinguished experts in this field, John detailed protein stabilization in the areas of aqueous solution during freezing and freeze-thawing, as well as during dehydration. Useful discussions afterward complemented his inspiring talk.

LEUKOCARE's 8<sup>th</sup> Scientific Formulation Symposium will take place on September 22, 2020. If you would like to participate, please contact [bd@leukocare.com](mailto:bd@leukocare.com). We look forward to seeing you in 2020.