Program Outline for—

Advancing Manufacture of Cell and Gene Therapies VI

Keynote talks and technical sessions will be organized into the following streams:

Session 1 – Advances in cell processing: New Techniques for new therapies
Session Chairs: Jeff Chalmers (Ohio State University) and
Tom Heathman (Hitachi Chemical Advanced Therapeutics Solutions)

The cell therapy industry has advanced rapidly from demonstrating promising clinical data to achieving commercially approved products. Due to the unprecedented speed of this development, several challenges remain in the manufacture of these cell therapy products that must be resolved through development of new technologies and solutions from across our entire community. In this session, we will explore potential cell therapy technologies that can resolve these challenges.
We invite abstract submissions focusing on:
1) Novel product platforms for next generation cell therapies (e.g. iPSCs, T-cells, gene-modified CD34+, MSCs and exosomes);
2) New upstream processing technology (e.g. bioreactors, xeno-/serum-free culture media, cell selection, and scale-up/scale-out of patient-specific/off-the-shelf products);
3) New downstream processing technology (e.g. cell separation, formulation and fill, cryopreservation and point-of-care thawing); and
4) New technology to automate, integrate and execute closed cell therapy processing.

Session 2 – Engineering challenges of *in vivo* gene therapy
Session Chairs: Fernanda Masri (Sartorius Stedim) and
Michael Greene (Novartis)

Gene therapy has rapidly advanced over the last few years thanks to the creation and application of sophisticated gene-editing technologies. These technologies have brought significant advantages such as targeted gene insertion and improved safety profiles. As a consequence, and strengthened by shorter regulatory approval times, commercial development and time to clinic have seen rapid acceleration. This session aims to address recent advances in viral vector production, emerging gene-editing tools and their deployment in human clinical trials.

In this session, we invite abstracts that address topics such as:
1) The manufacturing and scale up of established viral vector technologies (e.g., lentivirus), as well as next generation gene-editing tools (e.g., CRISPR/Cas9);
2) The clinical application and associated safety and efficacy profiles;
3) Limitations and technology gaps in the current state of the art, along with potential emerging solutions.

Session 3 – Gene-modification of cells for therapy

Session Chairs: Sean Palecek (University of Wisconsin-Madison) and
Phil Bassett (Adaptimmune Ltd.)

Genetic modification of cells offers the potential of designing cells for immunotherapy and regenerative medicine applications. In recent years, advances in gene editing precision have revealed the true potential of cell therapies. To realize the full potential of cell therapies, more efficient and precise methods are needed to introduce exogenous or modify endogenous genes in the human genome.

In this session, we welcome abstracts that:

1) Describe recent advances in gene modification of cells for therapy. Specific areas that could be explored include viral vector production; nonviral gene editing; and synthetic biology approaches to engineering therapeutic cells to improve their safety and potency.
2) Describe novel strategies for manufacturing and subsequent applications of gene-edited therapeutic cells.

Session 4: Product Characterization and Analytics

Session Chairs: **Damian Marshall** (Cell and Gene Therapy Catapult) and
Eric Rutjens (Novartis)

As the cell therapy industry moves towards closed, automated processing and more product enter clinical development there is an increased requirement for tools and technologies to allow better control over the manufacturing process. This opens-up exciting opportunities to look at the how frameworks such as process analytical technologies (PAT) can be applied to improve product manufacture, how in-process analytics can be applied to support real time product release and how new analytical strategies can be developed to support adaptive manufacturing.

In this session, we invite abstracts that address some of the latest approaches to product characterization and associated analytical techniques. Topics of particular interest include:

1) New biosensors or technologies for in-process monitoring;
2) Application of advanced techniques for high level product characterization;
3) Technologies to allow feedback control during manufacturing;
4) Advancements towards real-time testing for cell therapy products; and
5) Development of better potency assays to allow fast, safe release of products.

Session 5: Big data, analytics and control strategies

Session chairs: **David Pollard** (Kite Pharma) and
Krish Roy (Georgia Institute of Technology)

The continued efforts to drive down costs and improve efficiency of cell therapy process development and manufacturing require multiple, integrated IT strategies. This session will discuss big data strategies such as digital lab solutions for process development as well as electronic batch record/automated data capture for clinical and commercial manufacturing. Examples could include automation and real time process monitoring to speed problem solving and improve process robustness, while utilizing the latest process analytical technology. In addition, data mining capability and AI approaches across the cell manufacturing process can enhance identification of CQAs and CPPs and could be key to enhancing quality assurance. Data mining and AI can enable improved process understanding of complex

interactions, such as the relationship of the patient incoming apheresis to the manufacturing performance and clinical outcome. The integration of data from genomic and metanomic approaches will also be discussed for the further progression of truly personalized approaches.

Session 6: Bioprocess modeling for successful commercialization of advanced therapies

Session Chairs: **Jon Gunther** (Juno Therapeutics) and
Suzanne Farid (University College London)

Key to the success of the advanced therapies sector are cost-effective manufacturing and sustainable commercialization models. Bioprocess modeling efforts have a critical role to play in enhancing predictive insight as well as providing line-of-sight to feasible business models.

This session invites papers that:

1) Discuss insights from bioprocess modeling, simulation and optimization tools such as chemometric and agent-based modeling;
2) Present applications of such modelling approaches with industrially-relevant case studies addressing manufacturing and supply chain decisions for cell and gene therapies are particularly encouraged. This can include techno-economic feasibility for achieving optimal cost of goods (COG) at the commercial scale, simulation models to enhance facility design and capacity management across global sites for scale-up and scale-out scenarios, asset strategies optimization, business models for point-of-care versus centralized distribution for global deployment, the financial impact of process choices on the product development lifecycle, and chemometrics for enhanced process understanding and root cause analysis.

Session 7: Revolutionizing/Delivering the pipelines

The cell and gene therapy industry has advanced at an incredible speed over the last decade and now embraces a wider spectrum of advanced methodologies targeting several hematological malignancies and rare diseases, with powerful efficacy. Patients are benefiting from these new therapies thanks to advances in fundamental underpinning science, new translational tools and technologies, adaptation of flexible regulatory frameworks and introduction of accelerated access pathways. This represents tremendous success for the industry but what are the current limitations in delivering these medicines to mainstream healthcare?

In this final session, we invite abstracts that:

1. Focus on the vision for delivering the pipelines of emerging cell and gene products over the next decade.
2. Focus on addressing how these new medicines will be delivered in the context of mainstream healthcare. Themes that might be explored include transformation of supply chain logistics; clinical infrastructure for delivery to patients; synergizing parallel services for end-to-end manufacture.