FOREWORD

Spotlight on: the cell & gene therapy manufacturing pathway

Chris Mason & Elisa Manzotti

The cell and gene therapy sector has recently seen an influx of investment and interest from Big Pharma and venture capital companies, in part due to the promising early clinical data from immunotherapies such as CAR T cells and engineered T cells. However, a number of key questions and challenges remain on the path to successful commercialization.

Working with our expert Guest Editors – Dr Gregory Russotti (Celgene Cellular Therapeutics, USA), Dr Stephen Ward (Cell Therapy Catapult, UK), and Dr Peter Zandstra (Univ. Toronto, Canada) – we have identified the critical issues along the manufacturing pathway and these will be discussed across a 4-part Spotlight Series in 2016.

The four part series will focus on:

1. Defining commercial attributes early in product development
2. Integration of manufacturing and delivery into healthcare systems
3. New approaches to process validation and supply
4. Advances in analytical toolkit development

As reflected by our Guest Editor experience, it is essential to engage key stakeholders not only from industry, but academia and government-funded institutions in working towards translational solutions, and this has been our intent with the selection of world-leading expert contributors.

Robert Deans (Rubius Therapeutics, Boston, USA) shares his extensive experience in mesenchymal stem cell manufacturing and reflects on what lessons can learned from a retrospective perspective that can be utilized to accelerate successful development of cell- and gene-based therapies today.

One of the crucial manufacturing considerations when moving your product towards therapeutic-scale production, is what process changes will be required and in turn, how might these changes impact the very nature of your product. **Gregory Russotti** (Celgene) discusses the need to have a thorough understanding of how the cell is intended to function as a therapy and detailed process characterization which elucidates how process parameters can affect product attributes.

Nicholas Medcalf (Loughborough University) discusses the dilemma cell-therapy start-ups face when taking steps towards commercialization: whether to opt for centralized or distributed manufacture. Nicholas talks us through the various advantages and disadvantages

to each approach and their potential impact on your business model.

Cost of goods (CoGs) is a term most people in the industry will be very familiar with, but not all will have an understanding of how to determine the impact manufacturing decisions might have on this critical metric. **Sarah Callens and colleagues** review their experience at the Cell and Gene Therapy Catapult of how different process development considerations can influence the manufacturing CoGs and provide a working example case study to illustrate the key CoG drivers.

Innovator Insight

Our **Innovator Insight** articles let us hear directly from companies central to the manufacture of cell and gene therapies – their perspective on how they are working to support the translation of this new therapeutic modality and sharing their experience to help foster debate and collaboration.

Cindy Collins discusses the approach GE Healthcare are taking to working with therapy providers, technology companies, and other stakeholders to tackle the real-life challenges faced by the industry.

We also gain insight from our interview with leading experts at **Lonza**, who discuss their extensive experience working with cell and gene therapy companies and highlight the challenges they typically work with clients to resolve across the manufacturing pathway.

Future Leaders showcase

We also kick off our **Future Leaders** showcase in which we invite emerging players in the field to share their perspective on critical issues facing the translation of cell and gene therapies. Throughout the year we will present insights from the next generation of business leaders and invite our readers to nominate individuals they wish to be considered. More details will be released shortly.

First up, **Qasim Rafiq** (Aston University, UK) discusses some of the considerations SMEs must face when seeking to fulfil the increasing demand for cells at a quality and quantity required for therapeutic application. Qasim also highlights that early-career researchers from both industry and academia with a strong grounding in translation research, are essential to accelerating the development process of cell and gene based therapies.

Our second profiled Future Leader is **Iwan Roberts** (Puridify, UK) providing his perspective on the bioprocess developments required to ensure the promise of cell-based therapies can be delivered in a safe, efficacious and affordable manner.

We hope you enjoy part one of the *Cell & Gene Therapy Insights* Manufacturing Spotlight – you can access all content free of charge on the CGTI website, in addition to a host of other useful content in our Manufacturing Resource Center.

Chris Mason
Senior Editor, Cell & Gene Therapy Insights, CSO AVROBIO

Elisa Manzotti
CEO and Founder, BioInsights
e.manzotti@insights.bio

DOI: 10.18609/cgti.2016.017