

## FOREWORD



# Welcome to *Cell and Gene Therapy Insights*

**Chris Mason & Elisa Manzotti**



The time is now for a truly multidisciplinary publication to provide a forum for debate and discussion by all stakeholders committed to progressing this field and converting innovation into new therapies for patients.

In the 12 months since the inception of *Cell & Gene Therapy Insights*, barely a week has gone by without some mention of a scientific advancement or investment in the space. Whilst the immunotherapy clinical data points to a transformative step-change in oncology treatment, the field of cell and gene therapy comprises an incredibly diverse range of therapeutic modalities and products, many of which remain at an early stage of development.

The challenges along the road to successful commercialization of a new cell or gene therapy span a multitude of facets – scientific, clinical, manufacturing, financial and regulatory – so the time is now for a truly multidisciplinary publication to provide a forum for debate and discussion by all stakeholders committed to progressing this field and converting innovation into new therapies for patients.

It is our intention that *Cell and Gene Therapy Insights* provides the forum in which these critical issues are addressed.

Our positioning as a translational journal is reflected in our exceptional Editorial Advisory Board – with expertise and experience in research, clinical trials, translation, manufacturing, commercialization, funding and regulation; we would like to thank them for their support for this exciting new venture.

### SHINING A LIGHT ON THE CRITICAL ISSUES – CGTI SPOTLIGHTS

To help address the critical issues facing those in the field, *Cell & Gene Therapy Insights* will feature a **Spotlight** section comprising a collection of themed articles tackling these topics and fostering discussion and debate.

In collaboration with our internationally-leading Editorial Advisory Board, we identify the key advances and challenges across the field and highlight these in our Spotlight feature with specialist content including articles, webinars and podcasts.

Our exciting line-up for 2015/16 includes:

- ▶ The future of gene editing and applications in cell & gene therapy
- ▶ Cell & gene therapy manufacturing pathways
- ▶ CAR-T cells
- ▶ The convergence of precision medicine and cell and gene therapy
- ▶ Developments in viral vector production
- ▶ Advances in iPS cell therapies

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### IN THIS ISSUE...

We kick things off with our inaugural Spotlight – **Navigating the Global Regulatory Landscape** – providing the latest updates on the regulation of cell and gene

therapies from three key territories: Japan, Europe and the USA.

As global Regulatory Agencies continue to evolve their policies to match the pace of development in cell and gene therapy, it is essential for both academic and commercial scientists to understand the implications of these changes on their path to translation and product commercialization.

In April 2015, the first stem cell product, Holoclar<sup>®</sup>, was granted a Marketing Authorisation in the European Union. Dr Paula Salmikangas (Finnish Medicines Agency, Helsinki, Finland) and colleagues discuss the regulatory and scientific framework for cell-based products in Europe and how this can be successfully applied in the development and evaluation of novel products.

Japan's pioneering and radical approach to regulating cell therapies and regenerative medicines is discussed by Shintaro Sengoku (Tokyo Institute of Technology, Japan) and colleagues who provide an overview of the recent changes and their perspective on the implications of this new framework. Lee Buckler (RepliCel, Canada) & Colin Novick (CJ Partners, Japan) share their insight into the commercial impact of these regulatory changes – not only within Japan but for international companies looking to collaborate or partner with Japanese companies to take advantage of this innovative regulatory environment.

In addition to the Spotlight content, *Cell and Gene Therapy Insights* also publishes original research, reviews and a host of other added-value content such as interviews, perspectives and conference reports.

Currently the only effective treatment for advanced liver disease is liver transplantation, with demand far exceeding supply and approximately 20% of patients dying whilst on the waiting list. Dr Paul Newsome and Reenam Khan (University of Birmingham, UK) review the key advances in cellular therapies for liver disease, and discuss the challenges that must be overcome before these discoveries can be translated into clinical practice.

Also helping you keep apprised of the latest developments in the field, Fabio D'Agostino (Newcastle University, UK) captures the key highlights from the ISCT 2015 Annual meeting. Our unique Conference Insight format not only provides an overview of the main sessions and presentations, but also lets you hear from the delegates and speakers themselves with our conference chat soundbites and interviews.

## ADDING VALUE IN PUBLISHING

At *Cell and Gene Therapy Insights*, we are very mindful of the time constraints our readers are under – as such, you can stay up to date with all the latest advances with our **Insights** collection. In every issue, we publish a selection of key updates:

**Clinical Trial Insight** – Dr Alexey Bersenev (Yale University, USA) provides an overview of the latest clinical trial information, augmented with his expert commentary on published results.

**Commercial Insight** – our expert panel, including Alan Boyd, (Boyd's, UK), Mark Curtis

(CCRM, Canada) and Rahul Sarugaser (Hemostemix, Canada) provide a critical overview of the sector's commercial developments – M&As, licensing agreements & collaborations, financial results, IPOs and clinical/regulatory updates; highlighting their top picks for the month and 'ones to watch'.

We also provide a roundup of the top research and clinical developments selected from our highly-read news stories published daily at [www.insights.bio/cell-and-gene-therapy-insights/](http://www.insights.bio/cell-and-gene-therapy-insights/)

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online hub featuring high quality, relevant, engaging and interactive content including live webinars, podcasts, White Papers, conference reports and daily news updates on key milestones such as spin-outs, financings, candidates entering the clinic, and approvals.

After two decades of painstakingly building the foundations, the results are beyond our wildest expectations with transformative therapies (and even cures) for a small number of significant unmet medical needs ranging from leukemia to hemophilia. But this is just a small indication of the power of cell and gene therapy. Today, we are quite rightly proud of our cell and single gene-based therapies and their power to treat

disease. However, over the next decade, gene and genome manipulation, on a par with computer programming and debugging, will become the norm thus opening up the potential for addressing so many more unmet medical needs and thus transforming the lives of millions of patients and their carers worldwide. This will only be achieved if there is readily available and relevant information sharing between all the stakeholder groups in a format that is easily accessible and understandable. Working closely with all the community, we at CGTI will strive to help enable the big future for cell and gene therapy by publishing cutting-edge material, helping break down barriers, passionately promoting discussion, moving with the field, and most importantly, listening to our readers. We hope you enjoy the inaugural issue of *Cell and Gene Therapy Insights* – we would be delighted to hear your feedback to ensure this is a truly collaborative venture.

### AFFILIATIONS

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