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Running concurrently with the Washington Vaccine Forum 2010



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**6<sup>TH</sup> ANNUAL  
EVENT**

- Dr Celia M. Witten**, Director, Office of Cellular, Tissue and Gene Therapies (OCTGT), CBER, US FDA
- Dr Mitchell Gold**, President & CEO, Dendreon Corporation
- Dr Chuck Wilson**, Vice President, Global Head of Strategic Alliances, Novartis Institutes for Biomedical Research
- Andrea Hunt**, Vice President of Cellular Therapies, Baxter Healthcare Corporation
- Stephen Potter**, Senior Vice President, Corporate & Business Development, Genzyme Corporation
- Dr John McNeish**, Executive Director, Regenerative Medicine, Pfizer, Inc
- Dr Christian K. Schneider**, Head of Division of EU Cooperation/Microbiology, Paul-Ehrlich-Institut & Chair, EMEA Committee for Advanced Therapies (CAT)
- Dr Alain Vertès**, Global Alliance Director, Roche Pharma Partnering
- Dr C. Randal Mills**, President & CEO, Osiris Therapeutics, Inc
- Stephen M. Kelsey, MD**, Executive Vice President & Chief Medical Officer, Oncology, Geron Corporation
- Edward Lanphier**, President & CEO, Sangamo BioSciences, Inc
- Leslie Wolfe, PhD**, Vice President, Technology Development, Cellular Therapies, Genzyme
- George W. Dunbar**, Chief Executive Officer, President, Chief Financial Officer & Director, Astrom Biosciences
- Paul J. Schmitt**, Managing Director, Novitas Capital
- Gil Beyen**, Co-founder & Managing Director (CEO), TiGenix NV
- Elona Baum, Esq.**, General Counsel, California Institute for Regenerative Medicine (CIRM)

- Dr Aaron T. T. Chuang**, Senior Scientific Leader, GlaxoSmithKline
- David Smith**, Head of Cell Therapy, Lonza
- Dr Jacqueline Corrigan-Curay**, Acting Director, Office of Biotechnology Activities, National Institutes of Health
- Dr Claudio Bordignon**, President & CEO, MolMed SpA
- James M. Wilson, MD, PhD**, Head, Gene Therapy Program, Department of Pathology & Laboratory Medicine, University of Pennsylvania, School of Medicine
- Robert C. Moen, MD, PhD**, President & CEO, Copernicus Therapeutics
- Mike Rice**, President, CEO & Chairman, BioLife Solutions, Inc
- Dr Chris Holloway**, Group Director of Regulatory Affairs & CSO, ERA Consulting Group
- Dr Debra S. Grega**, Executive Director, Center for Stem Cell & Regenerative Medicine, National Center for Regenerative Medicine
- Dr Jeffrey Ostrove**, President & CEO, Ceregene
- Christopher J. Calhoun**, Chief Executive Officer, Cytori Therapeutics, Inc
- Prannath Marrott, FRACP, MRCP**, Senior Vice President, Clinical Development & Regulatory Affairs, AnGes, Inc
- Dr Jane Lebkowski**, Senior Vice President, Regenerative Medicine, Geron Corporation
- Gregg Sando**, CEO, Cell Medica Limited
- Ed Field**, President & COO, Aldagen
- Dr David Urdal**, Chief Scientific Officer, Dendreon Corporation
- Stewart Craig, PhD**, Senior Vice President, Development & Operations, StemCells, Inc

- Dr Joshua M. Hare**, Professor of Medicine, Director of the Interdisciplinary Stem Cell Institute, Miller School of Medicine, University of Miami
- Kristin Comella**, Vice President of Research & Corporate Development, Bioheart, Inc
- Dr Gary J. McGarrity**, Executive Vice President of Scientific & Clinical Affairs, VIRxSYS Corporation
- Dr Geert-Jan Mulder**, General Partner, Forbion Capital Partners
- Eric Faulkner**, Senior Director, US Market Access & Reimbursement, RTI Health Solutions
- Dr Ram Mandalam**, President & CEO, Cellerant Therapeutics, Inc
- Sheila A. Mikhail**, Chief Executive Officer, NanoCor Therapeutics, Inc
- Dr Gabor M. Rubanyi**, Chief Scientific Officer, Cardium Therapeutics
- Dr Denise K. Gavin**, Division of Cellular & Gene Therapies (DCGT), Office of Cellular, Tissue & Gene Therapies (OCTGT), CBER, US FDA
- Dr E. Edward Baetge**, Senior Vice President & CSO, Novocell
- Professor Sander Van Deventer**, CSO, AMT BV
- Dr Dirk Büscher**, Vice President of Research & Development, Cellerix
- Dr Joyce Frey-Vasconcelis**, Executive Director, PharmaNet
- Dr Tracey Lodie**, Director, Stem Cell Biology, Genzyme Corporation
- Jai Pal Singh, PhD**, Chief Scientific Officer, Vice President of Research, St Joseph's Translational Research Institute
- Dr Andrew L. Pecora**, Chairman of the Board, Amocyte
- Dr Janneke Meulenberg**, CEO, ORCA Therapeutics

- Dr Sonia Skarlatos**, Deputy Director, Division of Cardiovascular Sciences, National Heart, Lung & Blood Institute, NIH
- Dr Dan Gincel**, Director, Maryland Stem Cell Research Fund, Maryland Technology Development Corporation (TEDCO)
- Marc S. Penn, MD, PhD**, Medical Director of the Cardiac Intensive Care Unit, Cleveland Clinic
- Dr Philip D. Gregory, D. Phil**, Chief Scientific Officer, Sangamo BioSciences, Inc
- Aby J. Mathew, PhD**, Senior Director, Strategic Relations & Senior Scientist, BioLife Solutions, Inc
- Dr Samuel G. Jacobson**, Professor of Ophthalmology & Director, Center for Hereditary Retinal Degenerations & Retinal Function Department, University of Pennsylvania School of Medicine
- Dr Doug Jolly**, Executive Vice President of Research & Product Development, Tocagen, Inc
- Dr Scott R. Burger**, Principal, Advanced Cell & Gene Therapy
- Dr Wilfried Dalemans**, CTO & Vice President, Regulatory Affairs, TiGenix NV
- Moya Daniels**, Senior Director, Regulatory Affairs & Quality Assurance, Osiris Therapeutics, Inc
- Dr Alan Boyd**, Managing Director, alan boyd consultants ltd
- Robert Shaw**, Technical Director, Ark Therapeutics
- Dr Liz Bui**, Director, Intellectual Property & Corporate Development, Novocell
- Dr Donna Skerrett**, Director of Medical Affairs, Angioblast Systems, Inc
- Dr Dara Kraitchman**, Associate Professor, Russell H. Morgan Department of Radiology & Radiological Science, Johns Hopkins School of Medicine
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## 7.30 Registration & buffet breakfast in the exhibition area

### MORNING PLENARY SESSION

#### How will cell therapies, gene therapies and tissue-engineered products fit in to a reforming healthcare picture? Examining the commercialization strategies of companies in pivotal trials

- What degree of benefit will they need to deliver to secure reimbursement?

#### 9.00 Chair's introduction

**Dr Andrew L. Pecora**, Chairman of the Board, Amorceyte

#### 9.10 Opening keynote address

##### Payer's perspective on cell and gene therapies as commercial entities

- What prices can traditionally high-value biologics expect to reach in the evolving healthcare marketplace, and what benefit level will be required to achieve them?
- Will functional improvement be enough to gain top prices, or will such therapies need to have an impact on mortality?
- How commercially feasible are 'personalized medicines' given ongoing healthcare reform?

**Eric Faulkner**, Senior Director, US Market Access & Reimbursement, RTI Health Solutions

#### 9.30 Questions & discussion

#### 9.35 Phase III presentations and roundtable Representatives of companies with cell and gene therapies and tissue engineered products in Phase III trials will discuss how they are preparing for the commercialization of their products

- What are their experiences with navigating the BLA/MAA processes in the United States and Europe?
- How are they developing their pricing and reimbursement strategies?
  - How do you value a one-stop treatment in an increasingly stringent healthcare climate?
- What preparations are they making for commercial-scale manufacture and delivery?
- How should companies in preclinical and early-phase clinical development be looking to build reimbursement considerations into their R&D strategy?

- What are the key considerations in preparing for the logistical side of commercialization of these products?
  - Are the global supply and distribution logistics in place for the roll out of these therapies? If not, what preparations need to be made?

#### Speakers:

**Dr Mitchell Gold**, President & CEO, Dendreon Corporation  
**Dr C. Randal Mills**, President & CEO, Osiris Therapeutics, Inc  
**Gil Beyen**, Co-founder & Managing Director (CEO), TiGenix NV  
**Dr Claudio Bordignon**, President & CEO, MolMed SpA  
**Mike Rice**, President, CEO & Chairman, BioLife Solutions, Inc  
**Kristin Comella**, Vice President of Research & Corporate Development, Bioheart, Inc

11.05 Morning coffee in the exhibition area

## FOLLOWED BY YOUR CHOICE OF 3 PARALLEL BREAKOUT SESSIONS:

### FOCUS SESSION 1

#### An in-depth examination of autologous and allogeneic cell therapy business models: What are the keys to achieving commercial viability?

##### Moderator:

**Stewart Craig, PhD**, Senior Vice President, Development & Operations, StemCells, Inc

#### 11.45 Solutions to challenges often encountered on the path to commercialization of cellular therapies

- Tissue procurement
  - Oversee donor selection and screening and the tissue acquisition process
  - Determine final donor eligibility
- Process/media development
  - Media optimization (testing over 80 different basal media we manufacture)
  - Scale-up from flasks to large vessels
  - Determining optimal cell seeding density
  - Optimizing feeding schedule and cell densities
  - Development of commercial-scale cryopreservation methods
- cGMP manufacturing
  - Autologous cells of varying shelf life for shipment to the USA and Europe
  - Allogeneic cells, including sourcing, production of cell banks, and expansion to product quantities.
  - Stem cell production via both master and feeder cell banks
- Freezing, packaging, shipping and distribution
  - Delivering cells to clinical sites
  - Managing large-scale distribution centers
  - Regulatory services

**David Smith**, Head of Cell Therapy, Lonza

#### 12.05 Questions & discussion

##### Case studies

#### What are the chief strategic considerations with, and limitations of, autologous and allogeneic cell therapy manufacturing processes?

#### 12.10 Novel autologous cellular therapeutics addressing billion dollar markets and unmet clinical needs

- Compelling clinical data and well defined regulatory paths
- Established manufacturing infrastructure that can be scaled

- Debunking the myths of autologous therapies: Gross margins, logistics and adoption

**Ed Field**, President & COO, Aldagen

#### 12.30 Unique opportunities for expanded autologous mixed cell products: Targeting unmet medical needs and solving the challenges of commercial-scale manufacturing

- Multiple chronic disease targets
- Scale out manufacturing and distribution logistics
- Reliability, efficiency and cost effectiveness of automated processes

**George W. Dunbar**, Chief Executive Officer, President, Chief Financial Officer & Director, Aastrom Biosciences

#### 12.50 Buffet lunch in the exhibition area

#### 2.00 Development and commercialization of a novel universal allogeneic cellular therapeutic

- Business considerations in developing an allogeneic cell therapy
- Manufacturing/distribution, clinical and regulatory issues
- Commercialization challenges: COGS, reimbursement and market adaptation

**Dr Ram Mandalam**, President & CEO, Cellerant Therapeutics, Inc

#### 2.20 Extending stability and maximizing yield of source material and finished products

- The critical role biopreservation plays in commercialization of new cell therapies
- Detrimental effects of delayed onset cell death
- Avoiding assay pitfalls
- Extending stability of source material and finished products
- Maximizing post-preservation viability and functional recovery of finished products

**Aby J. Mathew, PhD**, Senior Director, Strategic Relations & Senior Scientist, BioLife Solutions, Inc

##### Case studies

#### Centralized vs onsite cell therapy processing: What are the relative pros and cons of each option, and how does each fit into the business model?

#### 2.40 Centralized

- Safety considerations
- Regulatory considerations
- Speed to market
- How are we each addressing the practical process/manufacturing /distribution issues with commercial scale in mind? (Eg. cell storage)

**Dr C. Randal Mills**, President & CEO, Osiris Therapeutics, Inc

#### 3.00 How do you begin to develop a model for point-of-care therapeutic delivery, given that the traditional manufacturing/delivery model is centralized?

- Cytori, as one of the first commercial-stage cell therapy companies, will be used as a case study to illustrate how to:
  - Navigate the product design and development process to bring an autologous point-of-care device to market
  - Commercialize, as well as train and educate the medical community in the practice of, cell-based therapies
- The following critical considerations will be addressed:
  - Autologous or allogeneic: Commit to model ideally suited for cell source, its attributes, and anticipated costs for manufacturing and distribution
  - Parallel product development, manufacturing, and regulatory strategies
  - Corporate partners: The if, when, and how to manufacturing, development and commercialization collaborations

**Christopher J. Calhoun**, Chief Executive Officer, Cytori Therapeutics, Inc

#### 3.20 Panel discussion

- How do autologous and allogeneic models compare with regard to their utility for cell-therapy-gene therapy combination approaches (ex vivo cell therapy) and with regard to therapy-device combinations?

##### Panelist:

**Dr Scott R. Burger**, Principal, Advanced Cell & Gene Therapy

#### 3.40 Moderator's closing summary

3.45 Close of session followed by afternoon tea in the exhibition area

### OR | FOCUS SESSION 2

#### Gene therapy clinical update: Analyzing recent clinical success stories in key therapeutic areas to inform your trial design strategy

#### 11.45 Moderator's introduction

##### Reviewing the gene therapy field: Who's still playing and which are now the most advanced product candidates on a global basis?

- What are the highlights from the past 12 months?
- Who's in the running to make a success with gene therapy?
- Which new products are the ones to watch?
- Is there a common theme emerging yet as to what will make a successful gene therapy product?

**Dr Alan Boyd**, Managing Director, alan boyd consultants ltd

##### Case studies

#### Updates on the latest clinical trial designs and data demonstrating clinical benefit for gene therapies in development

#### 12.00 Phase I trial of leber congenital amaurosis due to RPE65 mutations by ocular subretinal injection of Adeno-Associated Virus gene vector

- One rare form of blindness among many - clinical and molecular perspective
- Pre-clinical proof-of concept
- Proving the human blindness has the defective pathway worth treating
- Understanding results of the trial required standard but also novel outcomes

**Dr Samuel G. Jacobson**, Professor of Ophthalmology & Director, Center for Hereditary Retinal Degenerations & Retinal Function Department, University of Pennsylvania School of Medicine

#### 12.20 ZFP Therapeutics – diabetic neuropathy

- ZFPs operate at the DNA level to drive an array of unique therapeutic outcomes
- Transcription factor technology enables regulation of therapeutic target gene expression
- Sangamo's lead ZFP Therapeutic candidate, SB-509, which is designed to upregulate VEGF-A, has demonstrated neuroprotective and neuroregenerative functions in two Phase II clinical studies in moderate and severe Diabetic Neuropathy (DN)
- Within DN, SB-509 represents a novel, first-in-class, disease-modifying therapy

**Edward Lanphier**, President & CEO, Sangamo BioSciences, Inc

#### 12.45 Questions & discussion

12.50 Buffet lunch in the exhibition area

#### 2.00 CNS

- Results of a Phase II controlled Parkinson's disease clinical study; failure to meet primary endpoint
- Further analysis of the clinical data: statistically significant secondary endpoints
- Scientific rationale for the results; defects in retrograde transport
- Ways to overcome the issues; new targeting methods and mitigating the placebo effect

**Dr Jeffrey Ostrove**, President & CEO, Ceregene

#### 2.20 Questions & discussion

#### 2.25 Case study

##### Lessons learned in clinical trials with DNA-based therapeutics in chronic myocardial ischemia (Generx™) and diabetic foot ulcers (Excellerate™)

- Therapeutic angiogenesis with Generx (Ad5FGF-4) for chronic myocardial ischemia
  - The Phase II/III AGENT-3 and -4 trials with intracoronary application of Generx in patients with chronic myocardial ischemia (angina) provided important insights for the design of pivotal Phase III trials regarding the optimal patient population and clinical endpoints
  - Recent preclinical studies support the use of certain small molecules aimed at optimizing transfection efficiency when used in combination with Generx
- Enhanced wound healing with Excellerate (Ad5PDGFB in Gene Activated Matrix) for non-healing diabetic foot ulcers
  - Preclinical studies and a Phase I/II clinical trial provided evidence for the safety and efficacy of Ad5PDGF-B when applied in a Gene Activated Matrix (GAM)
  - A Phase IIb clinical study (MATRIX) has just been completed, and the results will be presented at the meeting

**Dr Gabor M. Rubanyi**, Chief Scientific Officer, Cardium Therapeutics

#### 2.45 Questions & discussion

#### 2.50 Oncology

**Dr Claudio Bordignon**, President & CEO, MolMed SpA

#### 3.10 Questions & discussion

#### 3.15 Questions for the speakers & panel discussion Identifying and addressing the immunological barriers to the development and commercialization of gene therapies

- What are the potential solutions?
- What steps can be taken in R&D from the early stages?

#### 3.40 Moderator's closing summary

3.45 Close of session followed by afternoon tea in the exhibition area

### OR | WORKSHOP

(Highly interactive session for a maximum of 30 participants)

#### Providing practical insights into how to build a commercially focused cell/gene therapy or tissue engineered product company from the ground up

- What are the key business challenges you will face at each stage of R&D, and how can you prepare to meet them?

#### 11.45 Moderator's introduction

**Paul J. Schmitt**, Managing Director, Novitas Capital

#### 11.50 What fundamental business, clinical, regulatory and operational infrastructure is required, and at what stage should each aspect be developed/introduced?

- How do you decide which aspects will be outsourced and which will remain core competencies as your organization starts to develop?
  - How do you assess whether a potential outsourcing partner has the specific technical expertise required for novel cell and gene therapy technologies?

- Staffing issues: Understanding whom you will need, when you will need them, and how to source them

**Kristin Comella**, Vice President of Research & Corporate Development, Bioheart, Inc

#### 12.10 Questions & discussion

#### 12.20 Preparing for the regulator: What are the keys to successfully navigating the regulatory process?

- Understanding the logistics of commercial delivery and the regulatory implications involved
  - How to build these considerations in to early-stage R&D
- How can QC considerations be pushed further upstream in cell therapy R&D? Assessing various technology approaches with the potential to deliver the data that regulators and CMOs alike will require at later stages

**Moya Daniels**, Senior Director, Regulatory Affairs & Quality Assurance, Osiris Therapeutics, Inc

#### 12.40 Questions & discussion

12.50 Buffet lunch in the exhibition area

#### 2.00 Understanding the pathway to the clinic: How to match research data with clinical targets?

- What data should you have before moving to the next stage?
- Rigorous product characterization at each stage of development

- At what stage in the pathway to the clinic should you create the infrastructure to develop a commercially viable manufacturing process?
- Managing risk and cost while moving into clinical development

**Dr Janneke Meulenber**, CEO, ORCA Therapeutics

#### 2.20 Questions & discussion

#### 2.30 The importance of defining your product at an early stage: How can/should you build dosing, labelling and reimbursement strategies into your early-stage R&D decision-making processes and study designs?

- Cell therapy production protocols must optimise among product quality/potency, clinical convenience, regulatory approval and reimbursement objectives
- Personal cell therapies frequently involve highly focussed applications – is off-label use possible when the cell therapy manufacturer has direct knowledge of each patient?
- Seeing the big picture for reimbursement – the cell therapy is often only a part of an overall procedure which needs reimbursement approval

**Gregg Sando**, CEO, Cell Medica Limited

#### 2.50 Questions & discussion

#### 3.00 Entering and progressing through the clinic: What are the keys to controlling costs and cash-burn rate at each phase?

- How much do you know about your product?
- How much should you know about your product at each stage?
- The biotech casino: Resource allocation, doing more with less, and risk assessment
- Partnering is good but can be stressful
- Setting goals and hitting them is a cash flow issue

**Dr Doug Jolly**, Executive Vice President of Research & Product Development, Tocagen, Inc

#### 3.20 Questions & discussion

#### 3.30 Questions, discussion & Moderator's closing summary

3.45 Close of session followed by afternoon tea in the exhibition area

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### AFTERNOON PLENARY SESSION

#### What traditional and alternative funding options are now open to academics and companies seeking to translate preclinical cell/gene therapy and tissue engineered product candidates to Phase IIa and beyond?

#### 4.25 Short presentations & roundtable discussion

- What funding is available in the translational phase?
  - Where are the key inflexion points for value creation and how can biotech companies progress through them in the current financial climate?
- VC perspectives: What specific aspects of a cell therapy, gene therapy or tissue engineered product add value in our eyes currently?
  - What is the relative value of product scalability versus cell source at the moment?
- What are the opportunities for working with academia and the NIH to bridge the translational 'valley of death'?
  - What alternative funding options are out there?
- What are the pros and cons of receiving orphan drug designation for a cell or gene therapy product and how must one adapt the business model accordingly?
- What opportunities are consortia providing to bridge the translational funding gap?

- What novel funding and cost-saving initiatives are available through the EU/EMEA
  - How can companies from all regions of the world capitalize on them?

##### Panelists:

**George W. Dunbar**, Chief Executive Officer, President, Chief Financial Officer & Director, Aastrom Biosciences  
**Paul J. Schmitt**, Managing Director, Novitas Capital  
**Dr Geert-Jan Mulder**, General Partner, Forbion Capital Partners  
**Dr Ram Mandalam**, President & CEO, Cellerant Therapeutics, Inc  
**Dr Samuel G. Jacobson**, Professor of Ophthalmology & Director, Center for Hereditary Retinal Degenerations & Retinal Function Department, University of Pennsylvania School of Medicine

5.40 End of day 1 followed by a themed cocktail reception in the exhibition area

MORNING PLENARY SESSION

## How will big pharma drive the continuing maturation of the cell and gene therapy sector?

- 9.00 Chair's introduction**  
**Edward Lanphier**, President & CEO, Sangamo BioSciences, Inc
- 9.05 Short presentations & roundtable discussion**  
**Examining the ongoing involvement plans and priorities of big pharma and big biotech companies**
- What are our current and future plans/activities in the cell & gene therapy and tissue engineered product areas?
    - What have been the drivers behind recent investments in technology platforms and what are our plans for expanded development and application of these platforms?
  - How do we conduct due diligence on prospective partners?
    - What data do we need to see regarding potential licensing/partnering opportunities?
    - How do we conduct due diligence in the stem cell space?
  - What are the latest trends in terms of the balance between the various collaborative strategies that we are pursuing (Eg. taking options in early-stage companies vs. licensing later-stage compounds)?
  - What are latest tools being developed by big pharma with utility for the cell and gene therapy field? (Eg. development of assays for potency and cell tracking, latest technologies for cell and gene imaging in vivo and their

- applications in preclinical and clinical development, new tools for ES and IPS cell line development)
- What are big pharma's priorities moving forward with regard to stem cells as potential therapeutics in their own right versus their utilization as research tools?
- Speakers:**  
**Dr Chuck Wilson**, Vice President, Global Head of Strategic Alliances, Novartis Institutes for BioMedical Research  
**Stephen Potter**, Senior Vice President, Corporate & Business Development, Genzyme Corporation  
**Dr John McNeish**, Executive Director, Regenerative Medicine, Pfizer, Inc  
**Andrea Hunt**, Vice President of Cellular Therapies, Baxter Healthcare Corporation  
**Dr Alain Vertès**, Global Alliance Director, Roche Pharma  
**Dr Aaron T. T. Chuang**, Senior Scientific Leader, GlaxoSmithKline
- 10.05 Cell and gene therapy industry response**  
**What can you do to help mitigate the risk of potential pharma partners?**
- How to prepare for due diligence?
- Dr Liz Bui**, Director, Intellectual Property & Corporate Development, Novocell

- 10.25 Questions & discussion**
- 10.30 Panel discussion**  
**What is the optimal exit strategy for cell/gene therapy and tissue engineered product companies in the current climate? Weighing up the various options**
- M&A, including reverse mergers
  - Assessing the public markets' appetite for cell and gene therapy
- Panelists:**  
**Stephen Potter**, Senior Vice President, Corporate & Business Development, Genzyme Corporation  
**Dr Alain Vertès**, Global Alliance Director, Roche Pharma  
**Robert C. Moen, MD, PhD**, President & CEO, Copernicus Therapeutics  
**Paul J. Schmitt**, Managing Director, Novitas Capital  
**Dr Geert-Jan Mulder**, General Partner, Forbion Capital Partners
- 10.55 Morning coffee in the exhibition area**

## FOLLOWED BY YOUR CHOICE OF 3 PARALLEL BREAKOUT SESSIONS:

### FOCUS SESSION 1

#### Driving the product characterization and optimization of cell & gene therapies through scale-up

- 11.35 Moderator's introduction**  
**Dr Joyce Frey-Vasconcells**, Executive Director, PharmaNet
- 11.40 Regulator's perspective**  
**Knowledge is power - what are the next steps on the path to cell and gene therapy product characterization?**
- Product characterization is critical to successful product development
  - Minor components in a product can have a big impact
  - Understanding product composition and product attributes related to potency are necessary parts of product characterization
  - Developing the technology and/or other strategies to characterize products
- Dr Denise K. Gavin**, Division of Cellular & Gene Therapies (DCGT), Office of Cellular, Tissue & Gene Therapies (OCTGT), CBER, US FDA

**12.00 Questions & discussion**

#### What are the next steps for the cell and gene therapy sector in terms of driving progress in product characterization?

- 12.05 Gene therapy**  
**What characterization work can/should be done at preclinical/early clinical stages?**
- Characterization of vector construct; in vitro and in vivo proof of concept: quality control assay development, especially potency and, for viral vectors, assay for replicating virus
  - Standard assay for quantitation of vector and/or transgene product
  - Process development and cGMP manufacturing process and assays on how to detect vector and/or transgene in patients
  - Full documentation, including SOPs for all procedures
- Dr Gary J. McGarrity**, Executive Vice President of Scientific & Clinical Affairs, VlrXSYS Corporation

**12.25 Questions & discussion**

#### Cell therapy

##### Presentation & panel discussion

##### What is the impact of impurity on cellular product characterization and what can be done about it in practical terms?

- Exploring FDA requirements for cell purity and its impact on potency and identity of the actual therapeutic product
- What technologies are currently available to assist in further defining the therapeutic cell population?
- Given the current lack of standards in terms of cell therapy QC, what is the minimum that cell therapy companies can/should do with regard to QC and when?

**Speaker:**

**Dr Joyce Frey-Vasconcells**, Executive Director, PharmaNet

**1.10 Buffet lunch in the exhibition area**

#### OR Lunch Briefing Sponsored by:



#### Cell and gene therapy industry case studies exploring challenges in the scaling of processes from GLP to GMP, and then from pilot to commercial scale

- What can/should you do to optimize your product as you progress through R&D? How do you keep your optimization moving forward as you advance through each stage?
- How to scale up clinical production to commercial scale whilst maintaining product comparability?
- Clarifying the differences between Phase I GMP requirements and those for later phases: How must you adapt?

#### 2.20 Cell therapy case study

##### Production of cell therapies from clinical trial to commercial distribution

- Examples of how to establish GMP production, validation programs and quality testing for clinical trials using cellular therapies will be discussed
- Genzyme currently manufactures three cell therapies for commercial distribution worldwide, details will be given on the production and quality processes that need to be established will be reviewed

**Leslie Wolfe, PhD**, Vice President, Technology Development, Cellular Therapies, Genzyme

**2.40 Questions & discussion**

#### 2.45 Gene therapy case study

##### Optimizing production of DNA nanoparticles for various stages of product development – taking the process from the research lab through early stage clinical to large scale clinical non-viral gene therapy applications

- Advantages of non-viral gene therapy manufacture process during the scale-up process
- Understanding production concerns at a research batch scale level
- Applying research batch scale knowledge to larger scale production suitable for early stage clinical trials
- Scaling up production via a continuous flow manufacturing system
- Closed system manufacturing versus open system manufacture for cGMP
- From grams to kilograms – can the same process work for vastly different scales?

**Robert C. Moen, MD, PhD**, President & CEO, Copernicus Therapeutics

**3.05 Questions & discussion**

#### 3.10 Considerations and examples for automation and commercial scale-out of patient-specific cell therapy manufacturing

- Scale-out strategy and contrast to multiple patient process
- Automation drivers and contrast to conventional drug/biologic process
- Choices for automation
- Example approach
- Financial and regulatory considerations

**Brian S. Hampson**, Senior Engineering Fellow, Aastrom Biosciences, Inc

**3.30 Questions & discussion**

#### 3.35 Moderator's closing summary

**3.40 Close of session followed by afternoon tea in the exhibition area**

### OR | FOCUS SESSION 2

#### What breakthroughs are being achieved in defining the Mechanism of Action (MoA) of cell therapies as they advance towards and through the clinic?

- 11.35 Moderator's introduction**  
**Big pharma perspective**  
**Andrea Hunt**, Vice President of Cellular Therapies, Baxter Healthcare Corporation

**11.50 Questions & discussion**

#### 11.55 The essentials of commercializing a cell therapy: Start at the end and work your way back

- The importance of connecting the dots (define molecular through clinical pathology, define Mechanism of Action, define therapeutic, and define distribution and reimbursement)
  - Clinical trial development (the importance of early assessment of efficacy)
  - Commercialization (who will use it, how will they use it, who will make it and how will they distribute it, and who will pay for it and why)
- Dr Andrew L. Pecora**, Chairman of the Board, Amorcyte

**12.15 Questions & discussion**

#### Case studies

##### Cell therapy trial designs and strategies to identify and demonstrate potency and Mechanism of Action at each phase of clinical development

- 12.20 Harnessing dendritic cells for cancer vaccination**
- Dendritic cells can be derived in vitro from peripheral blood leucapheresis products or from human embryonic stem cells
  - Electroporation of dendritic cells with RNA can create antigen presenting cells capable of raising a peptide-specific immune response
  - Vaccination of patients with manipulated (electroporated) dendritic cells may induce low-level yet potentially clinical significant immune responses in vivo
  - Questions still exist around specification for release of cellular therapies for human administration, the dose required for therapeutic effect, choice of antigen, and monitoring of responses
- Stephen M. Kelsey, MD**, Executive Vice President & Chief Medical Officer, Oncology, Geron Corporation

**12.40 Questions & discussion**

#### 12.45 Explaining Phase I and II clinical observations: Utilizing product characterization and laboratory assays to develop potency assays and formulate Mechanisms of Action

- Cell therapy production protocols must optimise among product quality/potency, clinical convenience, regulatory approval and reimbursement objectives
  - Personal cell therapies frequently involve highly focussed applications – is off-label use possible when the cell therapy manufacturer has direct knowledge of each patient?
  - Seeing the big picture for reimbursement – the cell therapy is often only a part of an overall procedure which needs reimbursement approval
- Dr Donna Skerrett**, Director of Medical Affairs, Angioblast Systems, Inc

**1.05 Questions & discussion**

**1.10 Buffet lunch in the exhibition area**

#### OR Lunch Briefing Sponsored by:



#### 2.20 Phase III case history: Development of Sipuleucel-T, an active cellular immunotherapy for prostate cancer

- Introduction to Sipuleucel-T
  - Characterization of the product and development of the potency assay
  - Clinical outcomes and correlation with product parameters
- Dr David Urdal**, Chief Scientific Officer, Dendreon Corporation

**2.40 Questions & discussion**

#### 2.45 Presentation & panel discussion

##### Defining the preclinical and clinical utility of the latest cell labelling and tracking technologies

- What technologies are available?
  - What are their relative pros and cons in terms of cost and potential benefit in providing biodistribution and cell fate data?
  - In what indications are they being implemented, and with what effect on preclinical and clinical development timelines and on clinical endpoints?
- Dr Dara Kraitchman**, Associate Professor, Russell H. Morgan Department of Radiology & Radiological Science, Johns Hopkins School of Medicine

#### 3.35 Moderator's closing summary

**3.40 Close of session followed by afternoon tea in the exhibition area**

### OR | WORKSHOP

(Highly interactive session for a maximum of 30 participants)

#### Regulatory 'surgeries'

These regulatory surgeries provide attendees with the opportunity to gain in-depth knowledge of the nuts and bolts of specific regulatory processes and procedures in the US, Europe and beyond, highlighting key regional differences and providing information on how to navigate around potential pitfalls and obstacles specific to the cell and gene therapy fields, and how to interact with regulators to ensure that clinical trial applications are successful. They will also provide attendees with the opportunity to ask detailed questions relating to their own activities in the area and receive expert advice.

**Moderator:**

**Dr Chris Holloway**, Group Director of Regulatory Affairs & CSO, ERA Consulting Group

#### 11.35 Surgery 1: Preparing for 'first in man' studies on gene and cell therapy products: Quality and nonclinical requirements for a Phase I Clinical Trial Application

- Regulations and guidelines: Which are helpful and what needs to be taken into account?
- Interacting with regulators at an early stage to validate quality and nonclinical requirements
- Where to conduct the 'first in man study', the US, Europe or elsewhere?
- The practicalities of compiling a clinical trial application for a 'first in man' study

#### 1.05 Moderator's closing summary

**1.10 Buffet lunch in the exhibition area**

#### OR Lunch Briefing Sponsored by:



#### 2.20 Surgery 2: Beyond Phase I: How to prepare for a successful clinical trial programme on a gene or cell therapy product

- Regulations and guidelines: What is applicable for a gene or cell therapy product, especially targeted at an 'unmet clinical need'?
- Interacting with regulators beyond Phase I to develop a successful clinical trial program
- Towards a marketing authorization application: Examining successes and failures (case studies)

#### 3.35 Moderator's closing summary

**3.40 Close of session followed by afternoon tea in the exhibition area**

### Gold Pass

A "Gold Pass" will allow you to attend the sessions of your choice at the Cell & Gene Therapy Forum and the co-located Washington Vaccine Forum. Contact [team@phacilitate.co.uk](mailto:team@phacilitate.co.uk) for more information.



#### 1.10 Lunch Briefing Sponsored by:



#### How non-invasive, in vivo imaging of transplanted cells is expected to accelerate the pace of cell therapy development, generate critical safety data and improve clinical outcomes

This session will outline which cellular imaging technologies are currently available for pre-clinical and clinical use, the anticipated costs and benefits of incorporating an imaging protocol into scientific discovery and/or clinical trials, and the anticipated impact of clinical imaging data on discovery and preclinical research, regulatory submissions, and clinical protocols

**Chair:**

**Charles F. O'Hanlon**, President & CEO, Celsense, Inc

**Speakers:**

**Robert Deans, PhD**, Senior Vice President, Regenerative Medicine, Athersys, Inc

**Eric T. Ahrens, PhD**, Associate Professor, Department of Biological Sciences, Carnegie Mellon University (Session for a maximum of 50 participants)

AFTERNOON PLENARY SESSION

## How are regulatory frameworks and guidelines for cell/gene therapies and tissue-engineered products continuing to evolve?

- Delivering the latest updates from the US and Europe

#### 4.20 Chair's introduction

**Moya Daniels**, Senior Director, Regulatory Affairs & Quality Assurance, Osiris Therapeutics, Inc

#### 4.25 US FDA perspective

##### FDA regulation of cellular, tissue, and gene therapies

- Overview of Office and regulation
- Update on recent guidances and meetings
- Challenges in regulation of cellular, tissue, and gene therapies

**Dr Celia M. Witten**, Director, Office of Cellular, Tissue & Gene Therapies (OCTGT), CBER, US FDA

#### Update on the European Committee for Advanced Therapies (CAT) on its first birthday: What are the experiences to date from both CAT and industry perspectives?

#### 4.45 Regulator's perspective

##### First experiences from the CAT's work

- How the CAT operates
- Challenges with Advanced Therapies
- How the CAT interacts with stakeholders

**Dr Christian K. Schneider**, Head of Division of EU Cooperation/Microbiology, Paul-Ehrlich-Institut & Chair, EMEA Committee for Advanced Therapies (CAT)

#### 5.00 Industry perspective

##### EU approval of ChondroCelect: Meeting the regulatory requirements for a cell-based product

- EU regulatory framework
- Challenges in meeting the classical CMC requirements
- Clinical trial design
- Benefit – Risk assessment

**Dr Wilfried Dalemans**, CTO & Vice President, Regulatory Affairs, TiGenix NV

#### 5.10 Panel discussion

- New EU post-market safety surveillance legislation: What are the chief considerations for cell therapy, gene therapy and tissue engineered product companies?
- What are the key areas of disharmonization between North American and European cell and gene therapy regulatory processes and standards?
- Clarifying the roles moving forward of non-regulator overview authorities such as the RAC and their processes by which they interface and harmonize with the regulators
  - What do the RAC see as their role as more gene therapy clinical trials move to Phase III?

**Panelist:**

**Dr Jacqueline Corrigan-Curay**, Acting Director, Office of Biotechnology Activities, National Institutes of Health

**5.50 End of day 2 followed by a themed cocktail reception in the exhibition area**

MORNING PLENARY SESSION

**Picking the winners: Assessing the prospects of - and progress with - emerging technologies**

- What are the keys to accessing Government funding in order to help realize their potential?

**9.00 Chair's introduction**

**Leslie Wolfe, PhD**, Vice President, Technology Development, Cellular Therapies, Genzyme

**Emerging technologies and cell & gene therapy candidates**

- Picking winners from early stage cell and gene therapy pipelines: What novel technologies/indications/therapeutic areas are showing particular promise?

**9.05 Presentation & panel discussion**

**Induced pluripotent stem cells (iPS cells): What progress is being made in industry and academia in this field?**

- Debating pathways forward for this, and other such novel technologies of great promise: How can the errors made in the early days of gene therapy be avoided?

Moderator:

**Dr John McNeish**, Executive Director, Regenerative Medicine, Pfizer, Inc

Panellists:

**Dr E. Edward Baetge**, Senior Vice President & CSO, Novocell  
**James M. Wilson, MD, PhD**, Head, Gene Therapy Program, Department of Pathology & Laboratory Medicine, University of Pennsylvania, School of Medicine

**9.40 Case study**

**ZFP Therapeutics - exploiting DNA as a platform for drug development**

- ZFPs operate at the DNA level to drive an array of unique therapeutic outcomes
- Transcription factor technology enables regulation of therapeutic target gene expression
- ZFN mediated genome editing can add, correct or delete target genes across transformed, primary, stem cells and transgenic models
- Multiple Phase 1 and Phase 2 programs highlight novelty, flexibility and speed of ZFP Therapeutic drug development

**Dr Philip D. Gregory, D. Phil**, Chief Scientific Officer, Sangamo BioSciences, Inc

**10.05 Solutions to overcome barriers encountered in gene therapy trials using first generation technology**

- Review immune barriers to in vivo gene therapy
- Discuss issues that should be considered in the design of clinical trials
- Present strategies and data to improve vector technology

**James M. Wilson, MD, PhD**, Head, Gene Therapy Program, Department of Pathology & Laboratory Medicine, University of Pennsylvania, School of Medicine

**10.20 Case study**

**Advancing human embryonic stem cell based therapies to clinical development**

- Production requirements
- Preclinical study requirements
- Delivery requirements
- Clinical study design

**Dr Jane Lebkowski**, Senior Vice President, Regenerative Medicine, Geron Corporation

**10.35 Cardiac cell therapy: Current status, challenges, and future directions**

- Lessons from current clinical trials
- Challenges of autologous cell manufacturing, homing and engraftment for optimum therapy
- New directions to optimize efficacy for cardiac repair through pharmacological treatment and scaffolding of cells

**Jai Pal Singh, PhD**, Chief Scientific Officer, Vice President of Research, St Joseph's Translational Research Institute

**10.50 Questions for the speakers and panel discussion**

**11.05 Roundtable discussion**  
**Examining stem cell R&D funding opportunities at State and national government levels: What are the keys to securing this funding?**

- How is this funding helping the services sector to develop in step with the industry?
- What is the real opportunity in terms of the potential for industry to gain funding for the clinical development of stem cell therapeutics?

Panellists:

**Elona Baum, Esq**, General Counsel, California Institute for Regenerative Medicine (CIRM)

**Dr Dan Gincel**, Director, Maryland Stem Cell Research Fund, Maryland Technology Development Corporation (TEDCO)

**Dr Debra S. Grega**, Executive Director, Center for Stem Cell & Regenerative Medicine, National Center for Regenerative Medicine

11.40 Morning coffee in the exhibition area

FOLLOWED BY YOUR CHOICE OF 3 PARALLEL BREAKOUT SESSIONS:

FOCUS SESSION 1

**Delivering strategic solutions to the key cost control issues and risk management conundrums with cell and gene therapy products**

- How to minimize Cost of Goods (CoG)?
- Build or buy?
- How to efficiently source both capacity and materials?

**12.20 Moderator's introduction**

**What will be the main drivers for Cost of Goods at commercial scale for cell and gene therapies, and what can be done to affect them?**

- How to scale up to commercial levels whilst controlling Cost of Goods and reducing cost-per-unit sufficiently to make the product viable?
- How far down can the sector realistically drive CoG?
- Weighing up the various options to access sufficient quantities of donor cells for a scaled-up allogeneic cell therapy process
  - How to minimize the impact on your CoG

**Dr Scott R. Burger**, Principal, Advanced Cell & Gene Therapy

12.45 Questions & discussion

**12.50 Exploring the impact of CoG at commercial scale on pricing, reimbursement and marketing/supply strategies**

- How do you develop a sales forecast related to commercial scale GMP manufacturing?

**Sheila A. Mikhail**, Chief Executive Officer, NanoCor Therapeutics, Inc

1.10 Questions & discussion

1.15 Buffet lunch in the exhibition area

**2.25 "Make vs Buy": Considerations in supply chain management for development and delivery of cell-based therapeutics**

- What is the product, the manufacturing process and the reagents used therein?
- What is the scale, throughput & complexity?
- Core expertise & CMO capabilities
- "Horses for courses" for the reagents and the cellular product

**Stewart Craig, PhD**, Senior Vice President, Development & Operations, StemCells, Inc

2.45 Questions & discussion

**2.50 Addressing the challenges for gene therapy companies in accessing capacity/technology for large scale gene therapy production**

- What are the lessons to be learned from the recent establishment of a state-of-the-art commercial gene therapy manufacturing facility?
- Is the gene therapy field as a whole ready to take the step up to commercial scale in the same way that monoclonals did when they broke onto the market?
  - What are the advancements in terms of developing gene therapy manufacturing standards and how much work still needs to be done?

**Robert Shaw**, Technical Director, Ark Therapeutics

3.10 Questions & discussion

**3.15 Panel discussion**

**How should you plan in advance to avoid issues with the availability and supply of raw materials as you scale up?**

- How must industry and the services sector collaborate to address the current shortfall of raw materials supply at commercial scale for both cell and gene therapy products?

**3.35 Moderator's closing summary**

3.40 Close of session and of the Phacilitate Cell & Gene Therapy Forum 2010, followed by afternoon tea

OR | FOCUS SESSION 2

**Stem cell therapy clinical strategy and data update**

- How are leading candidates performing in the clinic?
- Addressing key fundamental questions regarding the sourcing and delivery of stem cells

**12.20 Moderator's introduction**

**Dr Debra S. Grega**, Executive Director, Center for Stem Cell & Regenerative Medicine, National Center for Regenerative Medicine

**Case studies**

**Delivering the latest clinical trial designs and data from MSC-based cell therapies in development**

**12.25 MSCs for immune modulation/suppression**

**A look at Mesenchymal Stem Cells (MSCs) as potential cellular immune therapeutics to treat autoimmune disease**

- Objective: The immunomodulatory properties of human and murine bone marrow – derived mesenchymal stem cells (MSCs) include suppression of T cell responses and reduction of key inflammatory mediators, such as IFN- $\gamma$  and TNF $\alpha$ . We postulated that MSC mediated immune suppression may provide benefit in the treatment of spontaneous T cell-mediated autoimmune diseases, such as type 1 diabetes
- Research designs and methods: Allogeneic murine MSCs were administered intravenously in the non-obese diabetic (NOD) model of spontaneous diabetes, either prior to (preventative protocol) or at the time of disease onset (therapeutic protocol) in order to study the amelioration of disease

- Results: Prophylactic delivery of allogeneic MSCs to pre-diabetic NOD mice delayed the onset of disease. Therapeutic treatment at the time of disease onset was effective in reversing disease, as measured by restoration of blood glucose levels to the normal range. These data suggest that MSCs are able to modulate an ongoing autoreactive immune response

- Benefits:
  - MSCs can effectively alter an autoimmune response
  - MSCs can ameliorate ongoing diabetes
  - Impact of MSC therapy on other auto-immune diseases

**Dr Tracey Lodie**, Director, Stem Cell Biology, Genzyme Corporation

12.45 Questions & discussion

**12.50 MSCs for repair**

**Mesenchymal Stem Cell therapy for ischemic heart disease: Preclinical and clinical results**

- Discuss the translational development of cell-based therapy for human heart disease
- Discuss mechanism of data of cell-based therapy from large animal models
- Discuss clinical trial design of cell based therapeutics for human heart disease

**Dr Joshua M. Hare**, Professor of Medicine, Director of the Interdisciplinary Stem Cell Institute, Miller School of Medicine, University of Miami

1.10 Questions & discussion

1.15 Buffet lunch in the exhibition area

**2.25 Case study**

**When and why does cell source matter? Adipose derived stem cells in the cure of peri-anal fistula**

- Choice of cell types: Mechanism of Action
- Choice of cell types: Autologous versus allogeneic use
- Choice of cell types: EMEA/FDA
- Choice of cell types: Manufacturing and scale of economy
- Choice of cell types: FTO versus own patents

**Dr Dirk Büscher**, Vice President of Research & Development, Cellerix

**2.45 Challenges in delivering cells to the heart**

- The challenges of delivery to the heart
- The challenges of clinical trial design for cardiovascular cell therapy
- The NHLBI-funded cell cardiovascular therapy network
- The new NHLBI Cell Consortium

**Dr Sonia Skarlatos**, Deputy Director, Division of Cardiovascular Sciences, National Heart, Lung & Blood Institute, NIH

**3.05 Short presentation & panel discussion**

**Clarification of the latest developments with the stem cell patent environment**

- What are the repercussions for your IP portfolio and how can you strengthen your position in this regard?
  - Because of the complexity of the regulatory pathway, data exclusivity will be an important aspect as well as patent protection
  - Cross-licensing third party IP is likely necessary

**Dr Liz Bui**, Director, Intellectual Property & Corporate Development, Novocell

**3.35 Moderator's closing summary**

3.40 Close of session and of the Phacilitate Cell & Gene Therapy Forum 2010, followed by afternoon tea

OR | WORKSHOP

(Highly interactive session for a maximum of 30 participants)

**Overcoming the remaining challenges with viral and non-viral delivery platforms for gene and RNAi therapies**

**Case studies**

**Examining the latest safety and efficacy data for varying viral and non-viral vector platforms in clinical development: How are they performing and what issues remain?**

**12.20 Moderator's introduction**

**Adenoviral/ AAV vectors**

- Long-term expression and therapeutic benefit of allogeneic tiparvovec (Glybera™)
- One time intramuscular administration of allipogen tiparvovec (Glybera™) results in long-term expression and a significant therapeutic benefit in patients with LPL deficiency
- The AAV-LPL manufacturing process has been optimized, is fully scalable and cGMP compliant.
- The mode of action will be discussed
- Long-term safety (up to 4 years of follow-up), biodistribution and vector shedding data are presented

**Professor Sander Van Deventer**, CSO, AMT BV

12.45 Questions & discussion

**12.50 Lentiviral vectors**

- Analyzing recent instances of lentiviral vector integration in patients: What are the most recent developments with these and how can the safety profile of lentiviral vectors be improved moving forward?
- Safety data base on patients treated in VIRxSYS Phase I and II clinical trials
- Total number of autologous CD4+ cells administered to patients
- Total number of lentiviral copies administered to patients
- Number of patients' years of safety
- Total number of lentiviral vectors produced under cGMP for these trials
- Integration profiles of lentiviral vectors in patients

**Dr Gary J. McGarrity**, Executive Vice President of Scientific & Clinical Affairs, VIRxSYS Corporation

1.10 Questions & discussion

1.15 Buffet lunch in the exhibition area

**2.25 Plasmid DNA**

**HGF plasmid: Efficacy and safety from early phase trials in Critical Limb Ischemia**

- The current management of Critical limb ischemia
- The properties of HGF plasmid and the role of angiogenesis
- Efficacy and Safety data from early phase HGF trials
- Future challenges

**Prannath Marrott, FRACP MRCP**, Senior Vice President, Clinical Development & Regulatory Affairs, AnGes, Inc

**2.45 What progress are siRNA therapeutics making in the clinic and how are targeted delivery issues being addressed?**

Speaker to be announced

**3.05 Short presentation & panel discussion**

**Update on a combination cell therapy-gene therapy product candidate in clinical development**

- What is the latest data for an ex-vivo cell therapy candidate in development?
- How are delivery platforms impacting both safety and efficacy and how are challenges with both aspects being addressed through clinical trial design and technology development/application?
  - What constitutes the actual 'product' in the realm of ex vivo cell therapy? Is it the cell? Is it the gene? Is it the process? What are the repercussions for the clinical and business models?

**Marc S. Penn, MD, PhD**, Director, Skirball Laboratory for Cardiovascular Cellular Therapeutics, Center for Cardiovascular Cell Therapy & Senior Medical Director, Emerging Businesses, Cleveland Clinic

**3.35 Moderator's closing summary**

3.40 Close of session and of the Phacilitate Cell & Gene Therapy Forum 2010, followed by afternoon tea

This is the full program as it stands on the date of printing. However, the agenda will continue to evolve as we approach the event, ensuring that only the most current and relevant topics are addressed in Washington this coming January. Please visit [www.phacilitate.co.uk/cgtherapy](http://www.phacilitate.co.uk/cgtherapy) at any time to get updated with the latest developments.



Gold Pass

A "Gold Pass" will allow you to attend the sessions of your choice at the Cell & Gene Therapy Forum and the co-located Washington Vaccine Forum. Contact [team@phacilitate.co.uk](mailto:team@phacilitate.co.uk) for more information.



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We are offering a strictly limited number of sponsorship and exhibition opportunities to companies seeking to raise their profile with this uniquely influential audience.

The exhibition at the 2009 Washington meeting sold out weeks before the event for the third year in a row. We are already well on the way to replicating that success, with optimal booth spaces running short. To receive more information on exhibiting, or to find out about available sponsorship options (ranging from full event, workshop and cocktail reception packages to minor sponsorships, such as documentation memory sticks, program booklets and conference room drops), please don't hesitate to contact

**Nicola McCall**  
Tel: +44 (0)20 7839 6137 or  
Email: [nicola@phacilitate.co.uk](mailto:nicola@phacilitate.co.uk)

**AND REMEMBER! As an exhibitor at this meeting, you will have equal access to delegates and speakers at both the Cell & Gene Therapy Forum 2010 and the co-located Washington Vaccine Forum 2010 - over 500 senior life science executives with the authority to impact your business!**

**"The best opportunity to meet decision-makers in the field of cell/gene therapy and vaccine development"**

Paul Cronin, Business Development Manager, ERA Consulting Group

**"Phacilitate provides not only one of the best conferences of the year in this sector, but also recognises the value of, and supports, B2B networking that is critical to our ROI for such events"**

Lee Buckler, Marketing Communications,  
Progenitor Cell Therapy LLC

**"The coordination of the Phacilitate team was seamless"**

Chrissie DeCesare, Marketing/Client Relations, VGXI Inc

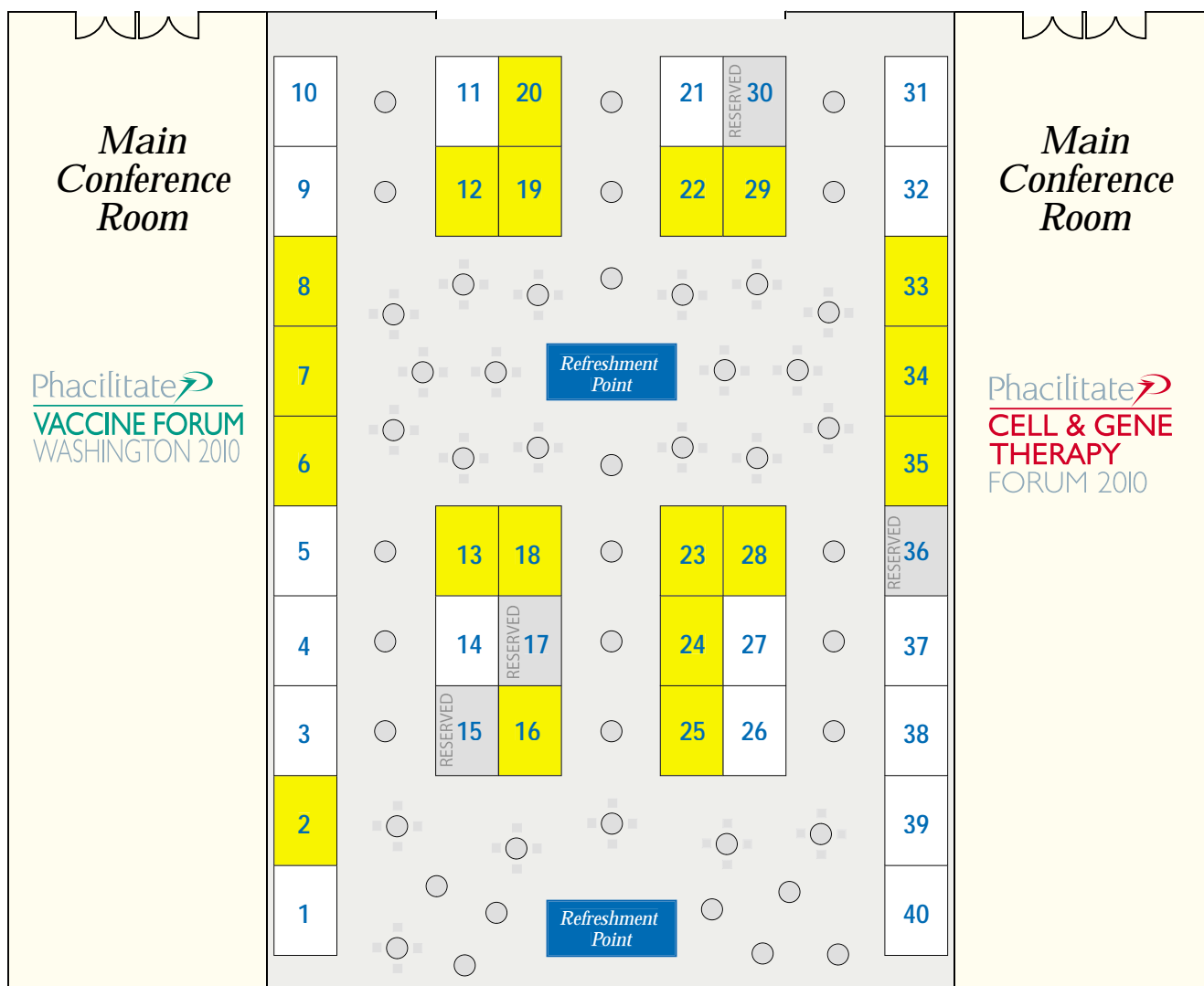
**"It really was a great event. I look forward to exhibiting again next year. Very informative and in a great setting that encouraged open dialogue and networking"**

Megan Barth, Director of Sales, Pharmaceutical & Biotechnology, Sterigenics

Silver sponsor:



Sangamo BioSciences, Inc. is focused on the research and development of zinc finger DNA-binding protein (ZFP) technology for therapeutic gene regulation and modification. By engineering ZFPs that recognize a specific DNA sequence, Sangamo has created ZFP transcription factors (ZFP TF(TM)) that can control gene expression and, consequently, cell function. Sangamo is also developing sequence-specific ZFP Nucleases (ZFN(TM)) for gene modification. The most advanced ZFP Therapeutic(TM) is currently in Phase 2 clinical trials in patients with diabetic neuropathy and ALS. Sangamo also has a Phase 1 clinical trial of a ZFN Therapeutic for the treatment of HIV/AIDS. Other therapeutic development programs are focused on cancer, neuropathic pain, nerve regeneration, Parkinson's disease and monogenic diseases. Sangamo has established strategic partnerships with companies in non-therapeutic applications of its ZFP technology, including Dow AgroSciences, Sigma-Aldrich Corporation, Genentech and Pfizer. For more information about Sangamo visit [www.sangamo.com](http://www.sangamo.com)



Exhibitors:

- |          |   |          |   |
|----------|---|----------|---|
| Booth 2  | <b>Regenerative Medical Enterprises</b> | Booth 22 | <b>DynPort Vaccine Company LLC, A CSC Company</b> |
| Booth 6  | <b>Meridian Life Science</b>            | Booth 23 | <b>Diosynth Biotechnology</b>                     |
| Booth 7  | <b>PharmaNet Development Group</b>      | Booth 24 | <b>BioLife Solutions, Inc</b>                     |
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The American Society of Gene & Cell Therapy (ASGCT) is a professional non-profit medical and scientific organization dedicated to the understanding, development and application of genetic and cellular therapies and the promotion of professional and public education in the field.

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 ■ facilitates the growth and development of the field of gene therapy through its scientific activities and its biennial meeting.  
 ■ represents the goals of the gene therapy community before government and the public  
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