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28-30 January,  
The Grand Hyatt, Washington, DC  
Running concurrently with the Washington Vaccine Forum 2008

# Phacilitate CELL & GENE THERAPY FORUM 2008

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Learn from - and network with - a unique panel of over 65 speakers. No other meeting for the sector attracts so many senior thought-leaders and decision-makers from the global cell and gene therapy industry and wider community - the individuals who are defining the R&D and product commercialization models of the future.

- **Earl M. Collier**, Executive Vice President, Genzyme Corporation
- **Dr Alan Sachs**, Vice President, RNA Therapeutics, Merck Research Laboratories
- **Dr Karen Kozarsky**, Head, Gene Therapy, Biopharmaceutical Center of Excellence for Drug Discovery, GlaxoSmithKline
- **Dr Naomi Aronson**, Executive Director, Technology Evaluation Center (TEC), BlueCross & BlueShield Association
- **Sudha Kadiyala, PhD**, Worldwide Director, Business Development & Strategic Planning, Johnson & Johnson Regenerative Therapeutics, LLC
- **Daniel Takefman, PhD**, Chief, Gene Therapy Branch, Division of Cellular & Gene Therapy, OCTGT/CBER, US Food & Drug Administration
- **Dr Akihiro Shimosaka**, Director, EPS Co. Ltd
- **David W. Levine, MD, MPH**, Vice President, Clinical Research, Genzyme Corporation
- **Dr John McNeish**, Senior Director, Pfizer Global R&D
- **Dr Mitchell H. Gold**, President & CEO, Dendreon Corporation
- **Professor Ryuichi Morishita**, Professor, Department of Clinical Gene Therapy, Osaka University Graduate School of Medicine & Founder, Board Member, AnGes MG
- **Professor Klaus Cichutek**, Vice President & Head, Division Medical Biotechnology, Paul-Ehrlich-Institut & Chair, EMEA Gene Therapy Working Party (GTWP)
- **Dr Jeffrey M. Ostrove**, President & CEO, Ceregene, Inc
- **Matt Kaplan**, Vice President, Senior Biotechnology Analyst, Punk, Ziegel & Co
- **Dr Peter Working**, Senior Vice President, R&D, Cell Genesys
- **Dr Garheng Kong**, General Partner, Intersouth Partners

- **Gerry Rodrigues PhD**, Principal Scientist, Biological Sciences RD3-2D, Allergan, Inc
- **Richard D. McFarland, MD**, Associate Director for Policy, OCTGT/CBER, US Food & Drug Administration
- **Kevin Sharpe**, Pan-European Biotech Analyst, ABN AMRO
- **John LeGuyader**, Director, Technology Center 1600, United States Patent & Trademark Office (USPTO)
- **Dr David Eckland**, Director of Research & Development, Ark Therapeutics
- **Eric C. Faulkner**, Director, US Market Access & Reimbursement, RTI Health Solutions & Director of the Genomics Biotech Institute of the National Association of Managed Care Physicians (NAMCP)
- **Dr Scott R. Burger**, Principal, Advanced Cell & Gene Therapy
- **Gabor M. Rubanyi, MD, PhD**, Chief Scientific Officer, Cardium Therapeutics, Inc
- **Dr Maria Palasis**, Director of Bioengineering, Boston Scientific Corporation
- **Carl H. June, MD**, Professor of Pathology & Laboratory Medicine & Director of Translational Research, Abramson Cancer Center, University of Pennsylvania
- **Dr Madhusudan V. Peshwa**, Vice President, Research & Development, Maxcyte, Inc
- **Dr Chris Holloway**, Group Director of Regulatory Affairs & CSO, ERA Consulting Group
- **Dr Jean-Yves Bonnefoy**, Vice President, R&D, Transgene SA
- **Dr Robert E. Sobol**, Senior Vice President, Medical & Scientific Affairs, Introgen Therapeutics Incorporated
- **Michael J. Werner**, President, The Werner Group (Founder & Board Member, Coalition for the Advancement of Medical Research & Former Chief of Policy, BIO)

- **Dr Barrie Carter**, Executive Vice President & Chief Scientific Officer, Targeted Genetics Corporation
- **Linda Powers**, Managing Director & Co-Founder, Toucan Capital Fund II & Chair, Maryland Stem Cell Commission
- **Dr Shelly Heimfeld**, Director, Cellular Therapy Laboratory & cGMP Cell Processing Facility, Fred Hutchinson Cancer Research Center
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- **Ed Field**, President & COO, Aldagen, Inc
- **Dr Gary McGarrity**, Executive Vice President of Scientific & Clinical Affairs, VIRxSYS
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- **Alain Rolland, Pharm D, PhD**, Senior Vice President, Product Development, Vical
- **Paul J. Schmitt**, Managing Director, PA Early Stage Partners

- **Ying Huang, PhD**, Pharmacologist, Pharmacology/Toxicology Branch, Division of Clinical Evaluation & Pharmacology/Toxicology, OCTGT/CBER, US Food & Drug Administration
- **Robert C. Moen, MD, PhD**, President & CEO, Copernicus Therapeutics, Inc
- **Dr Lincoln Tsang**, Partner, Arnold & Porter LLP, London
- **Doug Jolly, PhD**, President & COO, Advantagene
- **Elmar R. Burchardt, MD, PhD**, Vice President, Medical Affairs, Aastron Biosciences, Inc
- **Dr Mark O. Thornton**, Senior Vice President, Product Development, GenVec, Inc
- **Dr Sonia I. Skarlatos**, Acting Director, Division of Cardiovascular Diseases & NHLBI Gene Therapy Coordinator, NHLBI/NIH
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- **Theresa Chen, PhD**, Pharm/Tox Reviewer, OCTGT/CBER, US Food & Drug Administration
- **Dr Boro Dropulic**, CEO, Lentigen Corporation
- **Dr Alan Boyd**, Managing Director, alanboyd consultants ltd
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- **Dr Ram Mandalam**, Vice President of Pharmaceutical Operations, Cellarent Therapeutics, Inc
- **Dr Dirk Balshuesemann**, Project Manager, Cardiology, Miltenyi Biotec GmbH
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### Documentation

The conference documentation will be available on our website after the event for those individuals who cannot attend in person. If you are interested in receiving information on contents and cost in due course, please e-mail team@phacilitate.co.uk.

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MORNING PLENARY SESSION

## Defining current progress in - and key future target areas for - the global cell, tissue and gene therapy sectors

**Defining the global state of play in terms of cell, tissue and gene therapy candidates currently in phase II and III clinical trials - brief presentations outlining the leading product candidates in various geographical regions**

- 9.00 **Chair's introduction Europe**  
**Dr Alan Boyd**, Managing Director, alanboyd consultants ltd
- 9.15 **North America**  
**Dr Scott R. Burger**, Principal, Advanced Cell & Gene Therapy
- 9.25 **Asia/Oceania**  
**Dr Akihiro Shimosaka**, Director, EPS Co. Ltd
- 9.35 Questions & discussion

### Big pharma & big biotech keynote perspectives

- 9.45 **Big pharma perspective RNA therapeutic research and development capabilities at Merck Research Labs**
  - What are our current activities and near-term research plans?
  - How do Sima Therapeutics and Rosetta Inpharmatics (each wholly owned subsidiaries of Merck) contribute to our capabilities?
  - Which specific technologies are particularly attracting our interest?**Dr Alan Sachs**, Vice President, RNA Therapeutics, Merck Research Laboratories
- 10.05 Questions & discussion
- 10.10 **Big biotech perspective**  
**Earl M. Collier**, Executive Vice President, Genzyme Corporation
- 10.30 Questions & discussion
- 10.35 **Panel discussion Which therapeutic areas and specific indications will really drive progress in the cell and gene therapy sectors over the coming decade?**
  - Which applications have the greatest potential to achieve sustainable prices/ROI on R&D expenditure in the evolving therapeutics marketplace as a whole? Assessing the potential of, and competition in, key areas

- CV
  - Oncology
  - Neuro/CNS
  - Orthopedics
  - Ophthalmology
  - Diabetes
  - Does the convergence of cell therapy and gene therapy hold the key to enabling tomorrow's products to deliver sufficient efficacy and value to meet the increasingly stringent demands of the evolving healthcare marketplace?
  - In which area will the initial breakthrough be made?
  - What are the likely obstacles to technologies that utilize two (or more) potential synergistic therapeutic approaches, such as gene-modified cells?
  - What strategies are being developed for manufacturing of genetically modified cells?
- Panellists:** Speakers of the session, plus  
**Sudha Kadiyala, PhD**, Worldwide Director, Business Development & Strategic Planning, Johnson & Johnson Regenerative Therapeutics, LLC  
**Dr John McNeish**, Senior Director, Pfizer Global R&D  
**Linda Powers**, Managing Director & Co-Founder, Toucan Capital Fund II & Chair, Maryland Stem Cell Commission

11.05 Morning coffee in the exhibition area

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

### FOCUS SESSION 1

#### Delivering commercially viable processes and business models for the cell therapy sector

- 11.45 **Moderator's introduction Assessing scale-up issues at an early stage of process development**
  - Evaluate process operations that could be problematic
    - Select processes that will minimize scale-up issues
    - Design operating methods/equipment which demonstrated effective scale-up
  - Understand how process changes might affect biological activity
    - Comparability assessments
    - Potency assay(s)
  - Consider robustness/reliability of the manufacturing process
  - Compliance with current Good Manufacturing Production (cGMP) practices
    - Production process
      - Raw Materials
      - Equipment
    - Facility Requirements
      - Validation
    - Cost-effectiveness**Dr Shelly Heimfeld**, Director, Cellular Therapy Laboratory & cGMP Cell Processing Facility, Fred Hutchinson Cancer Research Center
- 12.05 Questions & discussion
- 12.10 **Update on Carticel® Exploring the ongoing product and process development strategies - lessons learnt from the commercialization of a cell therapy product**
  - Cell therapies have unique characteristics distinct from traditional pharmaceutical agents or medical devices that have important implications for all aspects of clinical development including trial design, manufacturing operations, regulatory approval, and reimbursement
  - Experience with Carticel® (autologous cultured chondrocytes) illustrates some of the approaches and expertise required to address these characteristics and bring a cell-based tissue engineering product into clinical practice
  - Carticel® demonstrates the feasibility of manufacturing a high quality cell product for surgical implantation and provides a foundation for further development of cell therapies
  - The Carticel® experience will be presented to illustrate the cross-functional strategy and infrastructure necessary to build this foundation for cell therapy programs**David W. Levine, MD, MPH**, Vice President, Clinical Research, Genzyme Corporation
- 12.30 Questions & discussion

**Case studies from companies in clinical trials with autologous and allogeneic cell therapies: What are the key challenges in processing and delivering these products to patients in the commercial setting and how are they being addressed?**

- What are the relative pros and cons of allogeneic versus autologous cell therapy models and how can they work from both business and regulatory standpoints?

- 12.35 **Autologous cell therapy**
  - Making the case for autologous cell therapies as viable products
  - What will be the issues in scaling up to the commercial level?
  - Weighing up the pros and cons of developing a model based on cryo-preserved vs. 'fresh' autologous cells**Ed Field**, President & COO, Aldagen, Inc
- 1.05 Questions & discussion
- 1.10 Buffet lunch in the exhibition area

**OR Working Lunch Electroporation technology applications in gene transfer**  
Moderator: **Drew Hannaman**, Vice President, R&D, Ichor Medical Systems, Inc (very informal, discussion-based optional session for a maximum of 12 participants)

- 2.20 **Allogeneic cell therapy How are we overcoming the practical obstacles to developing and implementing an allogeneic cell processing/distribution model?**
  - Cell sourcing, MCB/WCB production
  - Production and clinical supply logistics
  - Preclinical and clinical product experience
  - Clinical execution strategies
  - Barriers to international commerce and commercially viable solutions**Jack Harvey**, Director of Manufacturing, Pervasis Therapeutics, Inc
- 2.50 Questions & discussion

2.55 **Presentation reserved for Lonza Walkersville, Inc**

3.20 Questions & discussion

- 3.25 **Panel discussion**
  - Where training of physician/surgeons is needed, how can we persuade regulators to allow flexibility during clinical trials? To what extent can poor early results be viewed as a learning curve?
  - How do you introduce a quality control aspect where the physician plays an active role in the downstream processing of the therapy?
  - Will the centralized or localized model be dominant in the long run?

4.05 End of session followed by afternoon tea in the exhibition area

### OR | FOCUS SESSION 2

#### Overcoming the technical and regulatory barriers to the targeted delivery of gene-based therapies

- 11.45 **Moderator's introduction**  
**Dr Karen Kozarsky**, Head, Gene Therapy, Biopharmaceutical Center of Excellence for Drug Discovery, GlaxoSmithKline
- 11.50 **Case study Update on progress with an siRNA therapeutic candidate in clinical development**
  - Defining the specific delivery challenges - what major obstacles remain in terms of both safety and efficacy? How reliable and repeatable is the delivery process?
  - What are the assumptions for ongoing development and timelines for regulatory and commercial adoption?**Gerry Rodrigues PhD**, Principal Scientist, Biological Sciences RD3-2D, Allergan, Inc
- 12.15 Questions & discussion
- 12.20 **Panel discussion Debating the commonalities between siRNA and more 'traditional' gene therapies**
  - What lessons may each learn from the other?
  - Will the delivery of siRNA therapies drive the key first commercial breakthrough for gene therapy at large?
- 12.40 **Case study Adenoviral-based immunotherapy of cutaneous lymphoma**
  - Excellent safety data in clinical trials with non-replicative adenoviruses in human cancer trials
  - Passive immunotherapy for primary cutaneous lymphoma (CTCL, CBCL)
  - Phase I/II trial in CL
  - Medical need of novel therapies in CL**Dr Jean-Yves Bonnefoy**, Vice President, R&D, Transgene SA
- 1.05 Questions & discussion
- 1.10 Buffet lunch in the exhibition area

**OR Working Lunch Electroporation technology applications in gene transfer**  
Moderator: **Drew Hannaman**, Vice President, R&D, Ichor Medical Systems, Inc (very informal, discussion-based optional session for a maximum of 12 participants)

- 2.20 **Case study AAV vectors in clinical development**
  - Use of AAV vectors in clinical trials
  - Localized or systemic delivery
  - Disease targets and routes of delivery
  - Host responses
  - Safety profiles**Dr Barrie Carter**, Executive Vice President & Chief Scientific Officer, Targeted Genetics Corporation
- 2.45 Questions & discussion

- 2.50 **Case study Lentiviral vectors: Research tools, manufacturing applications, human gene therapy**
  - Development of highly efficient lentiviral vectors for gene silencing applications
  - Lentiviral vector mediated gene delivery as a platform for the manufacture of vaccines and biologics
  - Clinical translation of lentiviral vectors for human gene therapy**Dr Boro Dropulic**, CEO, Lentigen Corporation
- 3.15 Questions & discussion

- 3.20 **Case study Delivering the promise of nucleic acid therapeutics using non-viral nanoparticles**
  - Formulating nucleic acid nanoparticles that are stable, not immunogenic and nontoxic
  - Targeting of cell surface nucleolin
  - Examples of applications in lung, brain and eye
  - Clinical data in treating cystic fibrosis with DNA nanoparticles
  - Second generation targeted nanoparticles**Robert C. Moen, MD, PhD**, President & CEO, Copernicus Therapeutics, Inc
- 3.40 Questions & discussion

- 3.45 **Panel discussion Comparing and contrasting the performance of the various delivery platforms in terms of addressing traditional toxicity, specificity and re-administration issues**  
**Panellist:**  
**Theresa Chen, PhD**, Pharm/Tox Reviewer, OCTGT/CBER, US Food & Drug Administration
- 4.05 End of session followed by afternoon tea in the exhibition area

### OR | WORKSHOP

#### Quantifying market values and financing opportunities for the cell and gene therapy field on a global scale

Highly interactive workshop for a maximum of 30 participants

- 11.45 **Moderator's introduction**  
**Dr Jeffrey M. Ostrove**, President & CEO, Ceregene, Inc
- 11.55 **Wall Street analyst's perspective "Report Card" on the gene therapy sector for 2007**
  - How do investors and analysts perceive gene therapy as an investment opportunity, and why?
  - What hurdles are necessary for gene therapy to cross to attract a wider following?
  - Scorecard: A review of 2007 news flow (positive and negative) in the gene therapy sector and how it impacts perceptions**Matt Kaplan**, Vice President, Senior Biotechnology Analyst, Punk, Ziegel & Co
- 12.20 Questions & discussion
- 12.25 **Analyzing the investment climate for cell and gene therapy companies in Asia**  
**Dr Akihiro Shimosaka**, Director, EPS Co. Ltd
- 12.45 **Delivering the facts and figures regarding the commercial performance to date of a gene therapy on the market in China**  
Speaker to be announced
- 1.05 Questions & discussion
- 1.10 Buffet lunch in the exhibition area

**OR Working Lunch Electroporation technology applications in gene transfer**  
Moderator: **Drew Hannaman**, Vice President, R&D, Ichor Medical Systems, Inc (very informal, discussion-based optional session for a maximum of 12 participants)

- 2.20 **VC presentations & panel discussion A panel of VC firm representatives, all of whom are current or past investors in cell and/or gene therapy companies, will**
  - Discuss how their opinions on, and expectations for, the sector are evolving on an ongoing basis
    - To what extent are they willing to fund the sector now compared to in previous years?
  - Explain their criteria for assessing investment opportunities
    - What do they want to see from companies approaching them for investment?
    - What does an attractive investment prospect look like today?
      - Defining the key characteristics
    - How would they typically seek to structure funding based on the stage that the company has reached?
  - Reveal their views on pricing for cell and gene therapy products in the marketplace of the future.
    - What will the market be able to support?
    - How they are aligning their own ROI expectations with the overall trend of downward price pressure?**Speakers:**  
**Linda Powers**, Managing Director & Co-Founder, Toucan Capital Fund II & Chair, Maryland Stem Cell Commission  
**Dr Garheng Kong**, General Partner, Intersouth Partners  
**Gregory A. Bonfiglio**, Managing Partner, Proteus Venture Partners  
**Geert-Jan Mulder, MD**, General Partner, Forbion Capital Partners  
**B. Jefferson Clark**, Managing Partner, The Aurora Funds, Inc  
**Paul J. Schmitt**, Managing Director, PA Early Stage Partners  
**Antoun Nabhan**, Principal, Sagamore Bioventures
- 4.05 End of session followed by afternoon tea in the exhibition area

## REGISTER NOW AND TAKE THE OPPORTUNITY TO PROPOSE A TOPIC FOR A WORKING LUNCH SESSION



THEN AFTERNOON PLENARY SESSION

## Honing your ongoing business development strategy: What are the keys to attracting and retaining partners in the cell and gene therapy space?

- 4.45 **Chair's introduction Big pharma introductory overview**  
What are the chief incentives and the deal breakers for us in deciding whether or not to partner with a biotech company?  
**Sudha Kadiyala, PhD**, Worldwide Director, Business Development & Strategic Planning, Johnson & Johnson Regenerative Therapeutics, LLC
- 4.55 **Case study Examining a recent big biotech-gene therapy company deal**
  - What were the initial drivers behind the deal?
  - What are the keys to building and maintaining a successful, mutually beneficial relationship?**Dr Jeffrey M. Ostrove**, President & CEO, Ceregene, Inc

- 5.20 **Questions & panel discussion What degree of M&A and partnering activity can we expect to see in the event of cell and gene therapies products reaching the market in the West?**
  - What sort of strategies should cell and gene therapy companies adopt in this eventuality?
  - What will be the prime application areas for M&A activity?**Panellist:**  
**Douglas Doerfler**, President & CEO, MaxCyte, Inc
- 5.45 Close of day 1 followed by a themed cocktail reception in the exhibition area

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**Please Note:** These sessions are for limited numbers, once you have registered you will be able to reserve your place. REGISTER FOR THE CONFERENCE TODAY TO GUARANTEE YOUR PLACE IN THESE HIGHLY INTERACTIVE SESSIONS.

7.30 Registration & buffet breakfast in the exhibition area

## MORNING PLENARY SESSION

**What are the key trends in the ongoing evolution of the global regulatory environment for cell and gene therapies?**  
**• Delivering strategic action points for your organization**

### 9.00 Chair's introduction

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

### 9.05 US FDA perspective

**Regulatory perspective - ICH harmonization**  
**Daniel Takefman, PhD**, Chief, Gene Therapy Branch, Division of Cellular & Gene Therapy, OCTGT/CBER, US Food & Drug Administration

### Update on the European Advanced Therapies legislation

### 9.25 European regulator's perspective

#### EU regulatory aspects of Advanced Therapy Medicinal Products

**Professor Klaus Cichutek**, Vice President & Head, Division Medical Biotechnology, Paul-Ehrlich-Institut & Chair, EMEA Gene Therapy Working Party (GTWP)

### 9.45 Corporate strategy perspective

**Impact and consequences of the evolving regulatory framework for cell and gene therapy products from the industry perspective**

- Comparing and contrasting the regulatory framework for global development programs
- Fulfilling regulations and guidelines in the light of product diversity
- Practicalities towards developing a regulatory strategy to facilitate each stage of product development
- Case studies of some challenging issues in early-stage development of cell and gene therapy products

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

### 10.05 Questions for the speakers & panel discussion

- Highlighting key areas of harmonization and disharmonization between the US and European regulatory authorities
  - Orphan Drug Status
  - The definition of homologous versus non-homologous use of cells
  - What constitutes 'substantial manipulation' of a cell?

Panellist:

**Dr Shelly Heimfeld**, Director, Cellular Therapy Laboratory & cGMP Cell Processing Facility, Fred Hutchinson Cancer Research Center

### 10.25 Panel discussion

**Exploring the evolution of regulatory pathways in Asia and Oceania: What are the pros and cons for North American and European companies in terms of both clinical development opportunities and eventual market access?**

- China
- India
- Japan
- Singapore
- Australia/New Zealand

- What are the perceived regulatory challenges in moving a product from one region to another?

Panellists to include:

**Dr Christopher Savoie**, Founder, Representative Director & CEO, GNI Ltd

10.50 Morning coffee in the exhibition area

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

### FOCUS SESSION 1

## Optimizing your cell therapy and tissue regeneration product R&D, regulatory and scale-up strategies:

- What lessons may be learnt from companies currently in phase III clinical trials?

### 11.30 Moderator's introduction

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

### 11.35 Engineering cellular function: A paradigm shift in the development and manufacture of cellular therapeutics

This presentation will

- Demonstrate application of robust, scalable technology platform to specifically control cellular activity and function for cellular products to mimic in vivo requirements in diseases environment based on hypothesized mechanism of action of product in mitigating disease status
- Describe the ability to adequately characterize the cellular product and scale up the manufacturing process to clinical / commercial scale in compliance with quality & regulatory requirements
- Provide validation of approach using data from human clinical trials for engineered cellular products under investigation for use in immune disease and regenerative medicine applications

**Dr Madhusudan V. Peshwa**, Vice President, Research & Development, Maxcyte, Inc

12.00 Questions & discussion

### Case studies: Updates on cell therapy and tissue regeneration product candidates in phase III clinical development

- What are the latest clinical data and ongoing development and regulatory strategies?
- What are the key challenges they have faced, and are facing, in demonstrating sufficient efficacy to meet regulatory demands and in scaling-up their processes?
- How do you amplify anecdotal efficacy evidence gathered at the early stages in later trials?

### 12.05 Case study

#### Update on Provenge®

- Review of the latest data
- What has been the impact on ongoing development strategy of the approveable letter received from the FDA?
- What key lessons have been learnt to date in terms of both regulatory and clinical development strategy?

**Dr Mitchell H. Gold**, President & CEO, Dendreon Corporation

12.30 Questions & discussion

### 12.35 Case study

#### Update on ChondroCelect®

- Review of the latest clinical data
- Background and update on the clinical and regulatory strategy
- Manufacturing: Make or buy?

**Gil Beyen**, Co-founder & Managing Director (CEO), TiGenix

1.00 Questions & discussion

1.05 Buffet lunch in the exhibition area

### OR Lunch Briefing

(Optional session for a maximum of 50 participants) sponsored by

**Collaborating to accelerate the development of cellular therapies**



Presenter: **Stewart Craig, PhD**, Vice President & Chief Technology Officer, Progenitor Cell Therapy LLC

### 2.15 Manufacturing cell therapy products as clinical development progresses: Changes and comparability

- Bridging changes in manufacturing process/sites, analytical methods
- Managing manufacturing process scale/throughput
- Taking advantage of clinical experience
- Maintaining comparability/similarity

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

2.40 Questions & discussion

### 2.45 Case study

#### Challenges in development of a universal cell-based drug

- Prerequisites and advantages of universal cell based therapy
- Issues related to manufacturing and product characterization
- Addressing clinical and regulatory challenges early in the development process

**Dr Ram Mandalam**, Vice President, Pharmaceutical Operations, Cellent Therapeutics, Inc

3.05 Questions & discussion

### 3.10 Panel discussion

**In light of the experiences of companies in clinical development over recent years, how can we start to improve understanding of the Mechanism of Action/relevant biological function/potency and efficacy of a cell therapy at the preclinical stages to efficiently and effectively meet the evolving requirements of regulators?**

- What are the keys to enabling academia and the industry to work more closely together in achieving this goal?
- What novel technology areas have the potential to demonstrate efficacy at the early stages of R&D?
  - How, and at what stage, should cell-tracking technologies be introduced into product development and regulatory strategies?
  - In what instances might/should tracking data be required?

Panellists:

**Carl H. June, MD**, Professor of Pathology & Laboratory Medicine & Director of Translational Research, Abramson Cancer Center, University of Pennsylvania

**Ying Huang, PhD**, Pharmacologist, Pharmacology/Toxicology Branch, Division of Clinical Evaluation & Pharmacology/Toxicology, OCTGT/CBER, US Food & Drug Administration

4.00 End of session followed by afternoon tea in the exhibition area

### FOCUS SESSION 2

## Gene therapy clinical development strategy update: Defining the key benefits and pitfalls in conducting trials on a global basis

### 11.30 Moderator's introduction

**Dr Robert E. Sobol**, Senior Vice President, Medical & Scientific Affairs, Introgen Therapeutics Incorporated

### 11.35 Case study

#### Conducting a multinational gene therapy trial in Europe

- How have the obstacles arising from the inconsistency in the way that gene therapy products are delivered and released in different European countries been overcome? What practical lessons were learnt?
- Addressing the potential issue of negative perception of gene therapy by hospital pharmacists

**Dr David Eckland**, Director of Research & Development, Ark Therapeutics

12.00 Questions & discussion

### Case studies: What is the latest progress in the clinic for leading gene therapies from different therapeutic areas?

- What are the strategic keys to optimizing trial design and implementation strategies?
- Addressing scale-up and consistency issues on an international basis

### 12.05 Case study

#### Lessons learned from global (US and Europe) clinical development strategy (Phase 1 and II) for Ad2/HIF-1a/VP16, an angiogenic gene therapy

- The focus of the presentation will be on the Phase II clinical trial in patients with PAD (intermittent claudication) - the WALK study

**Kevin McEllin**, Senior Director, Clinical Research, Genzyme Cardiovascular

12.30 Questions & discussion

### 12.35 Case study

#### Lessons learned from the largest ever global angiogenic gene therapy clinical trial (AGENT) in patients with recurrent angina

- Based on promising Phase I and II clinical studies, the multinational Phase IIb/III trials, AGENT-3 and AGENT-4 were initiated to test the hypothesis that a single intracoronary injection of Ad5FGF4 will help alleviate recurrent angina in a broad patient population
- However, these trials, the largest global clinical trials so far of angiogenic gene therapy in a total of 532 patients, were discontinued based on an interim analysis of the AGENT-3 trial which predicted that it will not lead to significant benefits as performed
- Subsequent analysis of AGENT-3 data showed significant therapeutic effect only in a subpopulation of older patients with severe angina. In addition, pooled data from AGENT-3 and AGENT-4 revealed an unexpected gender specific effect of Ad5FGF-4 in the more than 80 women enrolled
- The new hypothesis, that angiogenic gene therapy may lead to clinically meaningful benefits predominantly in women with recurrent angina, is being tested in a recently launched Phase III clinical trial (AWARE)

**Gabor M. Rubanyi, MD, PhD**, Chief Scientific Officer, Cardium Therapeutics, Inc

1.00 Questions & discussion

1.05 Buffet lunch in the exhibition area

### OR Lunch Briefing

(Optional session for a maximum of 50 participants) sponsored by

**Collaborating to accelerate the development of cellular therapies**



Presenter: **Stewart Craig, PhD**, Vice President & Chief Technology Officer, Progenitor Cell Therapy LLC

### 2.15 Case study

#### Biomarkers in the development of oncology products

- Overview of FDA/NCI biomarker qualification initiatives
- Clinical biomarkers
  - PET scans for tumor response - Gleevec
- Molecular biomarkers
  - HER2/neu - Herceptin
  - Abnormal p53 - Advexin

**Dr Robert E. Sobol**, Senior Vice President, Medical & Scientific Affairs, Introgen Therapeutics Incorporated

2.40 Questions & discussion

### 2.45 Case study

#### Update on the GenVec pancreas cancer pivotal registration clinical trial with TNFerade (the PACT study)

- Review of the TNFerade study
- Design challenges with a rare cancer indication
- Keys to optimizing implementation of a gene therapy trial
- International disparities in oncology standard of care - a pitfall for global trials in pancreas cancer

**Dr Mark O. Thornton**, Senior Vice President, Product Development, GenVec, Inc

3.10 Questions & discussion

### 3.15 Panel discussion

**What are the criteria for successfully choosing outsourcing partners for both national and multinational gene therapy clinical trials?**

4.00 End of session followed by afternoon tea in the exhibition area

## THEN AFTERNOON PLENARY SESSION

**Addressing the key issues raised by the continuing convergence of the device sector with the cell, gene and tissue therapy sectors**

### 4.40 Short presentations & panel discussion

- What are the specific practical and regulatory challenges in defining the commercial business model for a product that incorporates a device?
  - How will these products be reimbursed? How is the issue of pricing, as a potential barrier to the commercial success of combination products, being addressed?
- Cell and gene therapy company and device company perspectives: What are the key competencies and skills required within each organization to enable them to work effectively with the other?
  - How do two companies from different sectors, and with different corporate cultures, work together effectively?
- Which risk-sharing models might be applied in the clinical development of combination products?
- What are the key (potential and existing) application areas for the

- complementary use of cell and gene therapies and medical devices moving forward?
- Will cell therapy or gene therapy lead the integration of these sectors?

Speakers:

**Richard D. McFarland, MD**, Associate Director for Policy, OCTGT/CBER, US Food & Drug Administration  
**Dr Joyce Frey-Vasconcelos**, Executive Director, PharmaNet Inc  
**Frank Corbin**, Vice President, Advanced Technologies, Gambio BCT  
**Elmar R. Burchardt, MD, PhD**, Vice President, Medical Affairs, Aastrom Biosciences, Inc  
**Eric C. Faulkner**, Director, US Market Access & Reimbursement, RTI Health Solutions & Director of the Genomics Biotech Institute of the National Association of Managed Care Physicians (NAMCP)

5.30 Close of day 2 followed by a themed cocktail reception in the exhibition area

### OR | WORKSHOP



## Exploring alternative funding mechanisms for cell and gene therapy companies

Highly interactive workshop for a maximum of 30 participants

### 11.30 Moderator's introduction

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

### 11.40 Update on US State stem cell funding initiatives - quantifying the opportunity for the cell therapy industry

- How can cell therapy companies obtain public capital?
- What is the status of stem cell funding across the US?
- Are states an appropriate and available source of funds for cell therapy companies?
- Does a stalemate in Washington mean there are no options?
- What is the financial picture beyond 2008?

**Michael J. Werner**, President, The Werner Group (Founder & Board Member, Coalition for the Advancement of Medical Research & Former Chief of Policy, BIO)

12.00 Questions & discussion

### 12.05 Analyst's perspective

#### Examining the IPO process - what are the keys to success?

- Feedback from European investors on recent gene therapy company IPO
- The difference between approaching a specialist and generalist fund manager
- What needs to be done pre-IPO to maximise the probability of success?

**Kevin Sharpe**, Pan-European Biotech Analyst, ABN AMRO

12.30 Questions & discussion

### 12.35 Industry IPO case study

**Dr Christopher Savoie**, Founder, Representative Director & CEO, GNI Ltd

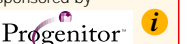
1.00 Questions & discussion

1.05 Buffet lunch in the exhibition area

### OR Lunch Briefing

(Optional session for a maximum of 50 participants) sponsored by

**Collaborating to accelerate the development of cellular therapies**



Presenter: **Stewart Craig, PhD**, Vice President & Chief Technology Officer, Progenitor Cell Therapy LLC

### Examining the benefits of, and keys to accessing, non-traditional sources of investment

### 2.15 NIH perspective

#### NHLBI/NIH resources for gene and cell-based therapies

- The presentation will include a description of the following resources and programs that are available to NHLBI/NIH investigators:
  - Gene Therapy Resource Program
  - Fetal Non-Human Primates Gene Transfer Center
  - New program announcements for gene therapy
  - Cardiovascular Cell-based Research Therapy Network
  - SBIR/STTR mechanisms for gene and cell-based research

**Dr Sonia I. Skarlatos**, Acting Director, Division of Cardiovascular Diseases & NHLBI Gene Therapy Coordinator, NHLBI/NIH

2.40 Questions & discussion

### 2.45 Foundation funding

#### Can venture philanthropy make a difference?

- Role of foundation venture philanthropy in early stage discovery and development
- The impact of venture philanthropy on drug pipeline for orphan diseases
- Success stories on the use of venture philanthropy for companies and diseases

**Dr Robert J. Beall**, President & CEO, Cystic Fibrosis Foundation

3.05 Questions & discussion

### 3.10 Case study: Asklepios BioPharmaceutical and NanoCor Therapeutics

- Funding and exit opportunities via indication-specific subsidiaries: NanoCor Therapeutics and Asklepios BIO DMD
- Utilizing venture philanthropy
- Securing funding through corporate strategic relationships

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

3.30 Questions & discussion

### 3.35 Panel discussion

4.00 End of session followed by afternoon tea in the exhibition area

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8.00 Registration & buffet breakfast in the exhibition area

## MORNING PLENARY SESSION

### On the road to commercialization: What preparations need to be made to enable the first licensed products to flourish in the marketplace?

#### 9.00 Chair's introduction

**Michael J. Werner**, President, The Werner Group (Founder & Board Member, Coalition for the Advancement of Medical Research & Former Chief of Policy, BIO)

#### 9.10 How will cell and gene therapies fit into the reimbursement system?

- Overview of current levels of reimbursement for different therapeutic areas and product classes in the US and Europe
  - What is the reality with regard to the reimbursement of Orphan Drug products?
- What will the price threshold be in the marketplace of the future, above which new therapeutic products will price themselves out of being reimbursable? What sort of benefit-risk balance could be supported?

**Eric C. Faulkner**, Director, US Market Access & Reimbursement, RTI Health Solutions & Director of the Genomics Biotech Institute of the National Association of Managed Care Physicians (NAMCP)

#### 9.40 Presentation reserved

#### Payer's perspective: How will the first cell and gene therapy products on the market in the US be assessed for reimbursement?

- What data will we look for to support the decision on how much we can pay?

#### 10.10 View from the Blue Cross Blue Shield Technology Evaluation Center (TEC)

- What is important to Blue Cross and Blue Shield Plan medical decision makers?
- What are the critical outcome measures and how good is the evidence?
- How will value and affordability shape benefit design and the market for new technologies?

**Dr Naomi Aronson**, Executive Director, Technology Evaluation Center (TEC), BlueCross & BlueShield Association

#### 10.40 Questions for the speakers & panel discussion

10.55 Morning coffee in the exhibition area

#### Keynote industry responses

#### Updates on the commercialization plans of a cell therapy and a gene therapy company, each in phase III trials

- What are their strategies for gaining licensure and securing reimbursement?
- How will they counter the ongoing price squeeze?
- What are their expectations in terms of the size of their respective markets and how will they access them in a commercially viable way?
- How can all stakeholders' needs, internal and external, be built into product development?

#### 11.35 Cell therapy perspective GVAX immunotherapy for prostate cancer, a cell-based approach to cancer therapy

- Design and endpoints of the on-going Phase III clinical trials
- Current strategies for addressing reimbursement issues
- The interplay of the natural history of the cancer, unmet medical need and market size assessments affecting the product development program

**Dr Peter Working**, Senior Vice President, R&D, Cell Genesys

12.05 Questions & discussion

#### 12.10 Gene therapy perspective HGF (Hepatocyte Growth Factor) as a first angiogenesis gene therapy drug

- Translation research from university to commercialization
- Results of a phase II trial in the USA and a phase III trial in Japan to treat patients with critical limb ischemia
- Current situation of Japanese biotech and the new Government policy, "Innovation 25"

**Professor Ryuichi Morishita**, Professor, Department of Clinical Gene Therapy, Osaka University Graduate School of Medicine & Founder, Board Member, AnGes MG

12.40 Questions & discussion

12.45 Buffet lunch in the exhibition area

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

### FOCUS SESSION 1

#### Clinical and regulatory strategy update on adult stem cell therapies and tissue regeneration products in the cardiac area

##### Case studies: Clinical updates on cardiac cell therapies and tissue regeneration products

- How are therapies derived from different cell sources, and utilizing differing delivery mechanisms/devices, progressing?
- What are the specific regulatory and scale-up challenges with each approach?

#### 1.55 Moderator's introduction

##### Case study

##### The repair of damaged myocardium with Cardiac Repair Cells (CRCs): Rationale for injecting megadoses of autologous stem and progenitor cells

- What evidence is there to support the safety and efficacy of autologous bone marrow-derived stem cells in cardiac diseases?
- What are the desirable characteristics of an engineered cell product for cardiac disease applications?
- What preclinical data support the use of CRCs in cardiac diseases?
- How can CRCs be utilized clinically for the treatment of chronic ischemic heart disease?

**Elmar R. Burchardt, MD, PhD**, Vice President, Medical Affairs, Aastrom Biosciences, Inc

2.20 Questions & discussion

#### 2.25 Mesenchymal Precursor Cells (MPCs) for cardiovascular diseases

**Dr Silviu Itescu**, CEO, Angioblast Systems, Inc

2.45 Questions & discussion

#### 2.50 Case study

##### Adipose derived regenerative cells in cardiac therapy, worldwide

- Update on adipose derived regenerative cells in cardiac clinical trials
- Regional challenges and opportunities
- Leveraging global opportunities in cell therapy

**Dr Kai Pinkernell**, Senior Director, Regenerative Cell Technology, Cytori Therapeutics, Inc

3.10 Questions & discussion

#### 3.15 Case Study

##### Use of adult stem cells for tissue regeneration in cardiology: Clinical and regulatory strategies

- Preclinical research results with CD133 selected stem cells
- Clinical results from pilot studies and phase I and II trials
- Cell isolation technology
- Manufacturing of cellular therapy products
- Medical device versus biological

**Dr Dirk Balshuesemann**, Project Manager, Cardiology, Miltenyi Biotec GmbH

3.35 Questions & discussion

#### 3.40 Panel discussion

##### What is the optimal form of delivery mechanism for cardiac cell therapy from strategic business, technical and regulatory viewpoints?

- What are the implications for
  - Toxicity?
  - Controlling dose and yield?
- How can you manage the effect instrumentation has on the effectiveness of the therapy?
- Does the business model change for a product that incorporates a device?

Panelist:  
**Dr Maria Palasis**, Director of Bioengineering, Boston Scientific Corporation

4.10 End of session and close of the Phacilitate Cell & Gene Therapy Forum 2008 followed by afternoon tea

### OR | FOCUS SESSION 2

#### Delivering the latest strategic solutions to the remaining issues in gene therapy product and process development

##### 1.55 Moderator's introduction

##### Clarifying the phase-specific product and process development requirements for gene therapy candidates

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

##### Case studies: Update on the latest progress with the process development and product characterization of viral vectored and plasmid-delivered gene therapy products

##### 2.10 Case study

##### Changing the production process for an AAV vector during clinical development: Implications for product development

- What are the drivers to change the production system?
- What are the consequences for
  - Preclinical studies?
  - Product characterization and Quality Control?
  - Clinical studies?

**Dr Janneke Meulenberg**, Director, Project Management, AMT BV

2.30 Questions & discussion

##### 2.35 Case study

##### Filing a BLA for a plasmid-based gene therapy

- How has the challenge of developing a potency assay been tackled?
- What are the key challenges in making the jump to commercial scale and how are they being addressed?
  - Comparability

**Alain Rolland, Pharm D, PhD**, Senior Vice President, Product Development, Vical

2.55 Questions & discussion

##### 3.00 Case study

##### Overcoming the challenges in creating an injectable formulation involving a lentiviral vector

- Favourable characteristics of lentiviral vectors include: Good transduction in certain cell types and non-dividing cells, favourable safety profile and prolonged gene expression
- Challenges for injectable lentiviral design include: trafficking to desired cell type in vivo, resistance to complement and readministration
- Targeting strategies include pseudotyping into other viruses and/or incorporating targeting sequences into the lentiviral vector envelope. Some data are available for VSV, HCV, baculovirus, among others
- Need for appropriate model systems that will be predictive of human administration

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

3.20 Questions & discussion

##### 3.25 Presentation & panel discussion

##### How do you make the decision of whether to contract your manufacturing out or do it yourself?

- How do you know which option is best for your organization and when is the optimal moment to decide one way or the other?
- What are the keys to evaluating potential outsourcing partners?

4.10 End of session and close of the Phacilitate Cell & Gene Therapy Forum 2008 followed by afternoon tea

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### OR | WORKSHOP



#### IP workshop: How much protection does your patent really provide?

Highly interactive workshop for a maximum of 30 participants

##### 1.55 Moderator's introduction

##### EU perspective on the legal aspects of regulatory data and patent life extension

**Dr Lincoln Tsang**, Partner, Arnold & Porter LLP, London

##### 2.10 Update from the US Patent Office USPTO: Patentability issues concerning the examination of genes, cells, and gene therapy

- Review of current practice and procedure
- Recent USPTO Notices affecting biotechnology examination practice
- Recent US case law affecting biotechnology examination practice

**John LeGuyader**, Director, Technology Center 1600, United States Patent & Trademark Office (USPTO)

2.30 Questions & discussion

##### 2.35 Gene therapy perspective

##### Strategies in finding a way through the patent and licensing maze in gene therapy: What do you need, when should you get it, and how much to pay for it?

- How do you find out what you need without breaking the bank?
- Prioritizing and coordinating licensing efforts: I may need 7 licenses for this product – now what?
- The licensing environment: Institutional agendas, timing and perception
- Some typical deal structures, deficiencies and difficulties

**Doug Jolly, PhD**, President & COO, Advantagene

2.55 Questions & discussion

##### Industry and legal sector perspectives on the evolving IP environment in the cell therapy area

##### 3.00 Legal perspective

##### What exactly can and can't you patent in the stem cell therapy field?

- Historical perspective on patenting stem cell-related inventions
- Comparison of national laws re such patents
- Recent and pending changes in relevant US patent laws and regulations
- Major US court decisions impacting this field
- What about the future?

**Dr Erich E. Veitenheimer**, Partner, Patent Counseling & Prosecution Practice, Cooley Godward Kronish

3.20 Questions & discussion

##### 3.25 Buttressing sandcastles: An industry perspective on how to build IP value against changing tides

- International IP opportunities – as US laws seem to be swinging against patent holders, the laws of other jurisdictions seem to be swinging in the other direction; which is good, given how international R&D, clinical, and commercialization efforts are
- Not all patents are created equal – the importance of composition of matter claims: targeted research
- Alternatives to patents – greater emphasis on know-how, trade secrets: also considering regulatory exclusivity, such as orphan drug designations
- Importance of lobbying efforts/legislative savvy – different jurisdictions, different agendas create many risks but also many opportunities

**Ken Stratton**, General Counsel, StemCells, Inc

3.45 Questions & discussion

##### 3.50 Panel discussion

##### Debating the key differences between the US patent system for cells and those in European and Asian countries

4.10 End of session and close of the Phacilitate Cell & Gene Therapy Forum 2008 followed by afternoon tea

### Comments from participants at last year's event included:

"Great range of topics. Excellent networking. Real case studies and perspectives most valuable"

Stephen Potter, Senior Vice President, Corporate Development, Genzyme Corporation

"A great forum providing a comprehensive overview of the key issues facing cell and gene therapy development"

Dr John St Clair Roberts, Vice President, Clinical & Regulatory, Intercytex



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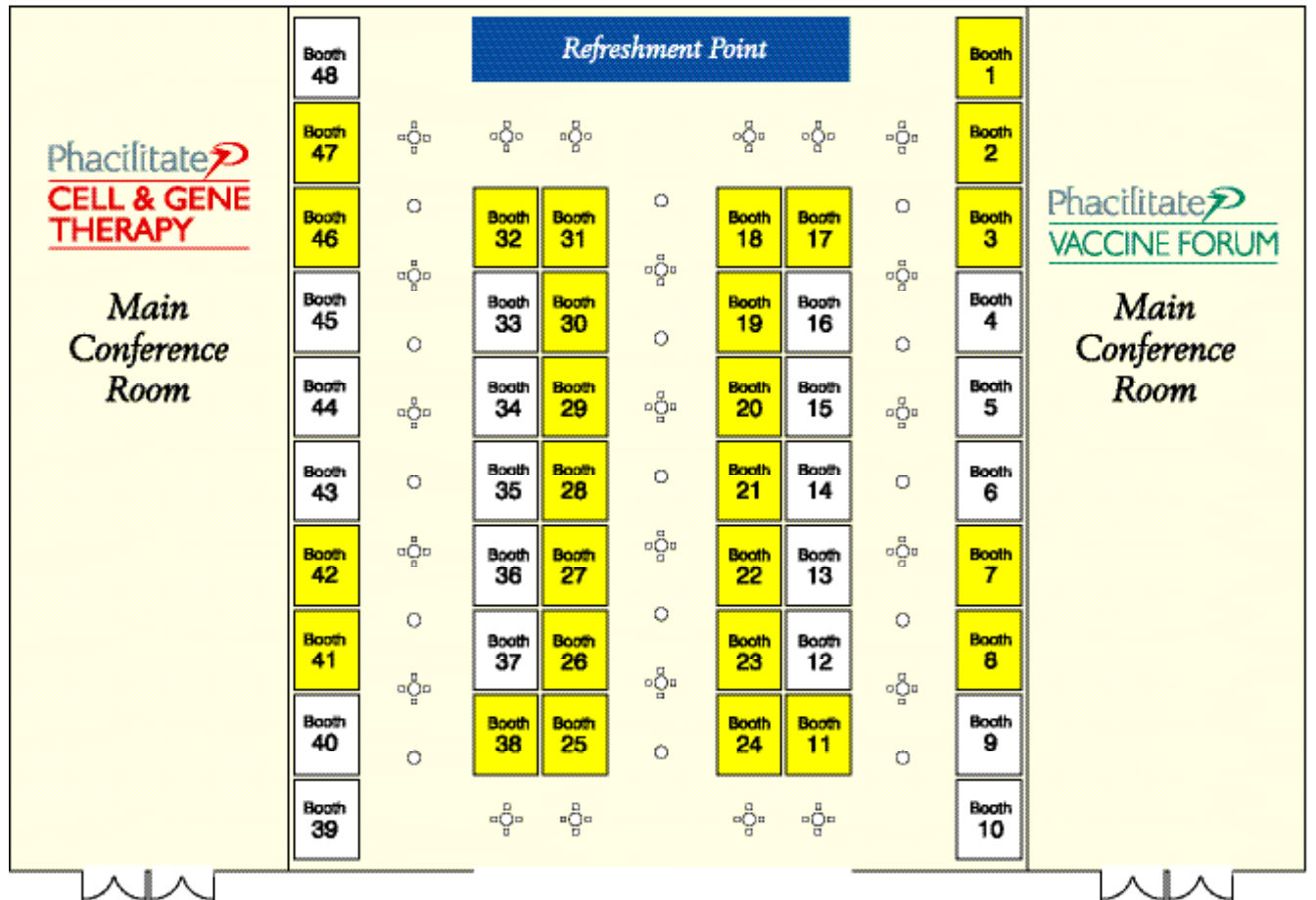
The exhibition at the 2007 Baltimore meeting was a tremendous success, with 100% of exhibitors stating on their evaluation forms that they achieved their major objectives in participating. We are already well on the way to selling out the 2008 floorplan - at the same point prior to the Baltimore '07 event, we had half as many confirmed exhibitors, and the floorplan ended up being sold out months before the meeting.

**"Another well-organized conference, with excellent networking opportunities with top-level decision-makers"**

Exhibitor, Dr May de las Alas, Business Development Associate, Ichor Medical Systems, Inc., speaking about her experience at the Baltimore 2007 meeting

As you can see, the best booth spaces are 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 conference documentation, delegate bags, event stationery and documentation inserts) please don't hesitate to contact Nicola McCall (t: +44 (0)20 7839 6137, or e: nicola@phacilitate.co.uk).

**AND REMEMBER! As an exhibitor at this meeting, you will have equal access to delegates and speakers from both the Cell & Gene Therapy Forum 2008 and the Washington Vaccine Forum 2008 - over 500 senior life science executives with the authority to impact your business!**



## Exhibitors:

- Booth 1 Aldevron
- Booth 2 McKenna Long & Aldridge
- Booth 3 Worthington Biochemical Corporation
- Booth 7 Covance
- Booth 8 PharmaNet
- Booth 11 Bridge Pharmaceuticals, Inc
- Booth 17 Cobra Biomanufacturing
- Booth 18 PRM
- Booth 19 AppTec
- Booth 20 Lonza Walkersville, Inc
- Booth 21 Lifeblood Biological Services, LLC
- Booth 22 Miltenyi Bioprocess
- Booth 23 Cognate BioServices, Inc.

- Booth 24 Pall Life Sciences
- Booth 25 DynPort Vaccine Company LLC, A CSC Company
- Booth 26 Biomedical Systems
- Booth 27 SAFC Pharma
- Booth 28 Ajinomoto AminoScience, LLC
- Booth 29 Thermo Scientific/HyClone Products
- Booth 30 BD Medical - Pharmaceutical Systems
- Booth 31 Emergent BioSolutions
- Booth 32 GE Healthcare
- Booth 38 Ichor Medical Systems
- Booth 41 ERA Consulting
- Booth 42 EUFETS AG
- Booth 46 MaxCyte, Inc
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MaxCyte is a clinical-stage company developing cellular therapeutics focused on oncology and regenerative medicine. The company uses its proprietary cell manipulation technologies to modulate and affect cell function. Additionally, MaxCyte licenses its technologies to partners for enablement of ex vivo cell therapies and for viral vector / biopharmaceutical manufacturing.

[www.maxcyte.com](http://www.maxcyte.com)



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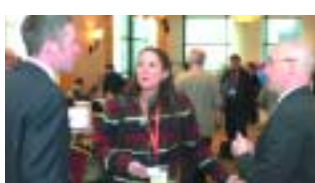


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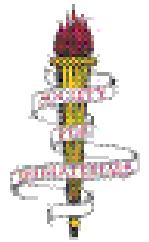
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