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- Dr Karen Kozarsky**, Head, Gene Therapy, Biopharmaceutical Center of Excellence for Drug Discovery, GlaxoSmithKline
- Dr Daniel Takefman**, Chief, Gene Therapy Branch, Division of Cellular & Gene Therapy, OCTGT/CBER, US Food & Drug Administration
- Dr Rajesh Chopra**, Global Medical Science Director, Emerging Products, AstraZeneca
- David G. Nance**, Chairman & CEO, Introgen Therapeutics, Inc
- Dr Sudha Kadiyala**, Worldwide Director, Business Development & Strategic Planning, Johnson & Johnson
- Stephen Potter**, Senior Vice President, Corporate Development, Genzyme Corporation
- Dr Mitchell H. Gold**, President & CEO, Dendreon Corporation
- Dr John McNeish**, Executive Director, Regenerative Medicine, Pfizer
- Shosh Merchav, PhD, MBA**, Director, Head, Cell Therapy Projects, Teva Innovative Venture, Teva Pharmaceutical Industries Ltd
- Edward Lanphier**, President & CEO, Sangamo BioSciences, Inc
- Dave Smith**, Head of Cell Therapy, Lonza Bioscience
- H. Stewart Parker**, President & CEO, Targeted Genetics Corporation
- Dr Gopalan Narayanan**, Manager & Head, Biologicals & Biotechnology Unit, Medicines & Healthcare Products Regulatory Agency (MHRA)
- Brock C. Reeve**, Executive Director, Harvard Stem Cell Institute

- Professor Ryuichi Morishita**, Professor, Department of Clinical Gene Therapy, Osaka University Graduate School of Medicine & Board Member AnGes MG
- Dr C. Randal Mills**, President & CEO, Osiris Therapeutics, Inc
- Gregory A. Bonfiglio**, Managing Partner, Proteus Venture Partners
- Muthiah Manoharan PhD**, Vice President, Drug Discovery, Alnylam Pharmaceuticals
- Dr Akihiro Shimosaka**, Director, Research & Development, Research Foundation for Community Medicine
- Peter X. Adams, MD, FACS, FCCP**, Medical Director, Cellular Therapies, Global Clinical Medical Affairs, Baxter BioScience
- Dr Jeffrey M. Ostrove**, President & CEO, Ceregene, Inc
- Kimberly A. Benton, PhD**, Deputy Director, Division of Cellular & Gene Therapies, OCTGT/CBER, US Food & Drug Administration
- John T. Dimos, PhD**, Senior Scientist, iZumi Bio Inc
- Dr Alan J. Lewis**, President & CEO, Novocell Inc
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- Paul J. Schmitt**, Managing Director, Novitas Capital
- Gary C. du Moulin, PhD, MPH**, Senior Director, Quality Compliance, Genzyme Biosurgery
- Dr Ann Tsukamoto**, COO, StemCells, Inc
- Dr Mark Scheideler**, Senior Scientific Officer, Roadmap Initiatives, National Institute of Neurological Disorders & Stroke, National Institutes of Health
- Dr Jane S. Lebkowski**, Senior Vice President, Regenerative Medicine, Geron Corporation
- Kim Raineri**, Director, Cell Therapy Operations, Lonza Bioscience

- Dr Stuart Naylor**, Chief Scientific Officer, Oxford Biomedica (UK) Ltd
- Henrik Ørum, MSc, PhD**, Vice President & CSO, Santaris Pharma A/S
- Bruce Wentworth, PhD**, Senior Director – Science, MG Biotherapeutics & Genzyme
- Dr Alan Boyd**, Managing Director, alanboyd consultants ltd
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- Professor Sander van Deventer**, CSO, AMT BV
- Dr Geert-Jan Mulder**, General Partner, Forbion Capital Partners
- Christine V. Sapan, PhD**, Executive Vice President & Chief Development Officer, Neurologix, Inc
- Dr Lincoln Tsang**, Partner, Arnold & Porter LLP, London
- Dr Doug Jolly**, Executive Vice President of Research & Product Development, Tocagen Inc
- Elyse Seltzer, MD**, Chief Medical Officer, Vice President, Clinical & Medical Affairs, Tengion
- Dr Scott R. Burger**, Principal, Advanced Cell & Gene Therapy
- Dr Gary McGarrity**, Executive Vice President of Scientific & Clinical Affairs, VIRxSYS
- Dr Krisztina Zsebo**, CEO, Celladon Corporation (pending final confirmation)
- Andrew L. Pecora, MD**, Chairman of the Board, Acting CEO, Amocyte
- Gregg Sando**, CEO, Cell Medica
- Bryan T. Butman PhD**, Senior Vice President, Vector Operations, GenVec, Inc

- Dr Weng Tao**, Chief Scientific Officer & Vice President, R&D, Neurotech
- Wei Liang, PhD**, Pharmacologist, Division of Clinical Evaluation & Pharmacology/Toxicology, OCTGT/CBER, US Food & Drug Administration
- Dr Christopher Bravery**, Director of Regulatory Affairs - Advanced Therapy Medicinal Products, ERA Consulting Group
- Robert C. Moen, MD, PhD**, President & CEO, Copernicus Therapeutics, Inc
- Anthony G. Coia, MD**, Principal, BioVentures Investors
- Dr Robert A. Preti**, President & Chief Scientific Officer, Progenitor Cell Therapy
- Dr Brett I. W. Zbar**, Principal, Aisling Capital
- Dr Michael Schuster**, Vice President, Operations, Angioblast Systems, Inc
- Marco Dieci, BSc**, Quality & Regulatory Director, Qualified Person, MolMed SpA
- Dr Andra Miller**, Director, Cell & Gene Therapies, Biologics Consulting Group, Inc
- Dr Kai Pinkernell**, Senior Director, Regenerative Cell Technology, Cytori Therapeutics, Inc
- Dr Wilfried Dalemans**, Vice President, Regulatory Affairs & Corporate Quality, TiGenix NV
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The conference fee does not include accommodation. We have negotiated substantially discounted rates for all attendees at the Cell & Gene Therapy Forum at The Grand Hyatt Washington. An accommodation booking form will be sent to you in your welcome pack, and you must use this form in order to qualify for the discounted rate which is only guaranteed for bookings received by the 5th of January 2009.

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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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7.30 Registration & buffet breakfast in the exhibition area

MORNING PLENARY SESSION

Maturing the cell, tissue and gene therapy sector - Creating and driving value along the R&D and commercialization path

9.00 Chair's introduction

Headline summary on status of approvals worldwide and on who is now closest to market

Dr Alan Boyd, Managing Director, alanboyd consultants ltd

9.10 **Big pharma's perspective on how the cell and gene therapy sector is reaching maturity and driving value**

• What is encouraging our interest in the sector?

Dr Ajan Reginald, Global Head of Emerging Technologies, Roche Group Research

9.30 Questions & discussion

Case studies from cell and gene therapy product pioneers: How are we driving value for all stakeholders, including payers?

- What practical advice do we have for those following us?
- When should you go to the payers and with what data?

- What reimbursement systems can be used for these therapies in the absence of classification codes?

9.35 Cell therapy

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

9.50 Gene therapy

David G. Nance, Chairman & CEO, Introgen Therapeutics, Inc

10.05 Questions & discussion

10.10 **A comparison with the commercialization path of monoclonal antibodies: What lessons can cell and gene therapy companies learn to avoid reinventing the wheel?**

- What elements of the overall maturation cycle will be the same, and which ones are truly unique to cell and gene therapy?

Eric C. Faulkner, Director, US Market Access & Reimbursement, RTI Health Solutions (*Invited*)

10.30 Panel discussion

Making money as a business and as an industry: How will value be driven?

- Therapies, tools and services: How is the sector developing and what needs to happen for all these layers to make money?
- Consolidation/mergers and acquisitions
- What is expected in 2009/2010?
- How are they an indication of market maturation?
- Investigating untapped synergies between regenerative medicine, tissue engineering, devices, and cell and gene therapy

Panelists:

Gregory A. Bonfiglio, Managing Partner, Proteus Venture Partners
Dr Brett I. W. Zbar, Principal, Aisling Capital

11.10 Morning coffee in the exhibition area

FOLLOWED BY YOUR CHOICE OF 3 PARALLEL BREAKOUT SESSIONS:

FOCUS SESSION 1

Manufacturing strategy: What roles are outsourcing, new platform technologies and process automation playing in achieving profitability?

11.50 Chair's introduction

How is supply and demand developing for cell and gene therapy contract production?

Dr John McNeish, Executive Director, Regenerative Medicine, Pfizer

Case studies

Making the decision to contract out manufacturing or retain in house

- How do you decide whether manufacturing is a core competence to keep in-house?
- Advantages/disadvantages to using a contract manufacturer instead of a partner pharma company
- Criteria for choosing a CMO: How do you assess the contactor's competence?
- How to manage the relationship successfully?
- Deciding whether testing should be outsourced or not

11.55 **Manufacturing recombinant adenovirus under cGMP conditions**

- Making the decision to contract out manufacturing or retain in house
- What criteria were used when choosing a CMO? What are the key elements of the contract?
- Assessing their competence for technology transfer, scale-up, CMC and validation of the manufacturing process
- Managing the relationship successfully

Bryan T. Butman PhD, Senior Vice President, Vector Operations, GenVec, Inc

12.15 Questions & discussion

12.20 **Cell therapy production on a global scale: Points to consider**

- Outsourcing each element of manufacturing – the good, the bad and the ugly
- Due diligence prior to acquisition of plants in Denmark and Australia
- Culture shock issues when integrating manufacturing from 3 continents
- Bringing organizations into compliance (past and present)
- Handling different regulatory requirements in each region
- Logistics issues, personnel and auditing issues

- Gap analysis utilization, risk assessment and critical quality parameters
- Harmonization in manufacturing and QC: Preventing Darwin's Finches
- Planning ahead for manufacturing in China

Gary C. du Moulin, PhD, MPH, Senior Director, Quality Compliance, Genzyme Biosurgery

12.40 Questions & discussion

12.45 Panel discussion

Manufacturing autologous and allogeneic products: How is cost of goods being controlled and what impact is regional manufacture having?

- How can we drive COGs down to profitable levels?
- How to manufacture regionally: Addressing logistical, shelf-life, storage and import/export considerations, and their impact on COGs
- What are the commercialization platforms for success?

Panelist:

Kim Raineri, Director, Cell Therapy Operations, Lonza Bioscience

1.20 Buffet lunch in the exhibition area

OR **Optional lunch workshop**

2.30 **Developing platform/enabling technologies: Which ones will be critical for the maturation of the sector by reducing cost of goods to make them reimbursable?**

- What progress is being made on the development of enabling technologies tailored to the needs of cell and gene therapy products?
- Ensuring that they are incorporated early enough to be able to support pivotal trials right through to commercialization

Geoff Mackay, President & CEO, Organogenesis Inc

2.50 Questions & discussion

2.55 Case study

What are the financial and technical implications of investing in automation?

- Do we understand the biology and mechanism of action well enough to use it?
- Can you do GMP using automation? What about validation?
- What impact does it have on COGs?

Penny Johnson, BSc, MSc, PhD, Director of Research, UK, Intercytex

3.15 Questions & discussion

3.20 Panel discussion

Debating the role of systems and process engineering in the manufacture of cell and gene therapy products

3.55 Moderator's closing summary

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

FOLLOWED BY AFTERNOON PLENARY SESSION

1.20 **Optional lunch workshop**

Biopreservation process optimization: Improving yield of source material and finished products

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OR | FOCUS SESSION 2

Designing cell and gene therapy clinical trials - Taking a close look at each phase of development to identify the key inflexion points, assess risk:benefit, and plan for commercialization and reimbursement

11.50 Chair's introduction

Dr Andra Miller, Director, Cell & Gene Therapies, Biologics Consulting Group, Inc

12.05 Translating from preclinical to phase I

- Translation of cell therapy to phase I: What does it take?
- Animal studies / toxicology models: What constitutes 'enough' animal testing?
- What are the issues associated with animal models?
- Cell banking and comparability

Dr Ann Tsukamoto, COO, StemCells, Inc

Cell therapy case studies

Illustrating the key elements of trial design and the coordination between clinical, manufacturing and commercial at each stage of the development process

12.25 Questions & discussion

12.30 Phase I/II blinded trial design

- Safety studies
- Dosage
- Collecting the right data all through the stages
- Providing biological assurance – mechanism of action

Andrew L. Pecora, MD, Chairman of the Board, Acting CEO, Amorcyte

12.50 Questions & discussion

12.55 **Peripheral blood derived CD34+ stem cells clinical trials in chronic myocardial ischemia subjects**

- Patient selection and recruitment
- Stem cell mobilization and selection
- Myocardial delivery systems
- Measurements of efficacy and safety outcomes
- Planning the pivotal study in CMI

Peter X. Adams, MD, FACS, FCCP, Medical Director, Cellular Therapies, Global Clinical Medical Affairs, Baxter BioScience

1.15 Questions & discussion

1.20 Buffet lunch in the exhibition area

OR **Optional lunch workshop**

Afternoon chair

Dr Karen Kozarsky, Head, Gene Therapy, Biopharmaceutical Center of Excellence for Drug Discovery, GlaxoSmithKline

Gene therapy case studies

Illustrating the key elements of trial design and the coordination between clinical, manufacturing and commercial at each stage of the development process

2.30 **Phase II trial design: What's the critical mass of patients required to demonstrate efficacy and support the commercialization path?**

- Dosage
- Collecting the right data for registration
- Efficacy: Demonstrating added value to patients and payers
- Link to commercialization and reimbursement

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

2.50 Questions & discussion

2.55 **The first clinical trial using lentiviral vectors: Findings from phase I/II and decisions regarding next steps**

- Successful delivery of a 937 base antisense sequence to HIV envelope
- Cumulative data on patient safety monitoring
- Coordination between manufacturing and clinical through the phases
- The next clinical trial: Phase IIb or phase III? Discussions with the regulator
- Defining the optimum patient population
- Re-imburement models

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

3.15 Questions & discussion

3.20 Panel discussion

Planning ahead for commercialization at every stage of the development process: What are the key criteria to consider at each inflexion point?

- Coordinating efforts across the organization to ensure that R&D, manufacturing and commercialization are fully supportive and in-step

Panelist:

Dave Smith, Head of Cell Therapy, Lonza Bioscience

3.55 Moderator's closing summary

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

FOLLOWED BY AFTERNOON PLENARY SESSION

AFTERNOON PLENARY SESSION

What's missing from the tool box?

- Understanding which tailored enabling technologies cell and gene therapy companies need to help get into humans and progress to market?

4.40 Panel discussion

Company perspectives: Which tailored enabling tools are missing and what would be first on the wish list?

- What types of matrix / scaffold would be valuable?
- What are the limitations of flow cytometry and how can we overcome them?
- High quality imaging to track vectors and cells
- Validated PCR to monitor cells – DNA & RNA
- Closed systems for cell extensions, cell separation and cell processing
- Use of small molecules in stem cell differentiation
- Potency assays & biomarkers
- Automation
- Adaptive trial design
- Development of full GMP (eg big pharma level quality)

- Fostering coordination between companies and tool/service providers to enable a better understanding of the needs of the cell and gene therapy sector

Panelists:

Dr Robert A. Preti, President & Chief Scientific Officer, Progenitor Cell Therapy

Ed Field, President & COO, Aldagen, Inc

Dr Ann Tsukamoto, COO, StemCells, Inc

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

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

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

OR | WORKSHOP

Latest results from the CNS area: How are cell and gene therapies progressing in preclinical and clinical development?

Highly interactive workshop for a maximum of 30 participants

11.50 Moderator's introduction

How are collaborations and partnerships with patient foundations and advocacy groups helping to drive these therapies into and through the clinic?

Dr Mark Scheideler, Senior Scientific Officer, Roadmap Initiatives, National Institute of Neurological Disorders & Stroke, National Institutes of Health

12.05 **Developing human embryonic stem cell based therapies for spinal cord injury**

- Rationale for therapeutic approach
- Production of hESC-based therapeutic cells
- Preclinical efficacy and safety studies
- Considerations in clinical trial design

Dr Jane S. Lebkowski, Senior Vice President, Regenerative Medicine, Geron Corporation

12.25 Questions & discussion

Gene therapy case studies

12.30 **rAAV-GAD: Targeting the Subthalamic Nucleus (STN) in Parkinson's disease patients**

- Parkinson's disease: The need for new therapies for medically refractory patients
- rAAV-GAD description and mechanism of action
- Phase I clinical study
 - Study design description
 - Study results and PET imaging study summary
- Phase II clinical study
 - Study design description
- Comparison between phase I & phase II study
- Preliminary safety results
- Perspectives on future clinical studies
- Combination product: The challenges, the obstacles and the lessons learned

Christine V. Sapan, PhD, Executive Vice President, Chief Development Officer, Neurologix, Inc

12.50 Questions & discussion

12.55 **Latest clinical results from Parkinson's disease and Alzheimer's disease programs**

- Parkinson's disease (PD) program
 - Controlled, multi-center phase 2 trial evaluating CERE-120 in advanced PD patients
 - Trial results expected early 2009
 - Long term follow all of all PD patients previously treated with CERE-120
 - Planning the initiation of a trial of CERE-120 in Europe
- Alzheimer's disease (AD) program
 - Safety data for the initial subjects treated with CERE-110
 - Enrollment of a higher dose study of CERE-110
 - Planning a controlled, multi-center trial in mild to moderate AD to be initiated early in 2009

Dr Jeffrey M. Ostrove, President & CEO, Ceregene, Inc

1.15 Questions & discussion

1.20 Buffet lunch in the exhibition area

OR **Optional lunch workshop**

Cell therapy case studies

2.30 **Application of encapsulated cell technology (ECT) for ophthalmic diseases**

- Description of the technology
- Advantage in treating chronic retinal degenerative diseases
- Preclinical and clinical development of ECT
- Future directions

Dr Weng Tao, Chief Scientific Officer & Vice President, R&D, Neurotech

2.50 Questions & discussion

2.55 **Novel T-cell vaccine being evaluated as patient-specific treatment for MS**

- Top-line data from the phase IIb study of Tovaxin®
- Specific tailoring to each patient's disease profile
- Efficacy, safety and tolerability results

Donna R. Rill, BS, MT (ASCP), Vice President of Operations, Opexa Therapeutics

3.15 Questions & discussion

3.20 Panel discussion

What is required to drive the CNS area forward to maturity?

- How can we make foetal tissue trials more robust?
- How can safety hurdles be addressed?

3.55 Moderator's closing summary

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

FOLLOWED BY AFTERNOON PLENARY SESSION

MORNING PLENARY SESSION

Analyzing the latest interpretation of the US, EU and Asian regulatory environments for cell, gene and tissue therapies - How are they impacting global R&D and commercialization strategies?

9.00 **Chair's introduction**

A comparison with the regulatory pathways for other biologics: Monoclonal antibodies, vaccines and RNAi

- What are the key similarities and differences?

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

How are regulatory pathways in US, Europe and Asia evolving?

9.10 **US**

- Update on international harmonization
- Update on approach to potency measurements
- Clarification on regulatory approach for ex vivo modified cells.

Dr Daniel Takefman, Chief, Gene Therapy Branch, Division of Cellular & Gene Therapy, OCTGT/CBER, US Food & Drug Administration

9.35 **EU**

- Role of CAT
- Role of the relevant EMEA working parties
- Innovation task force and its role
- Contribution of UK MHRA in EU
- MOA characterization
- Regulations concerning long-term follow up, in particular for vectors
- Regulation of combination cell/gene therapy products and devices
- What are the appropriate safeguards for clinical trials in fatal pediatric conditions?
 - Update on the obligation to insert a pediatric plan in your marketing authorization application
- Relevance and requirement for animal models

Dr Gopalan Narayanan, Manager & Head, Biologicals & Biotechnology Unit, Medicines & Healthcare Products Regulatory Agency (MHRA)

10.00 **Japan/Asia**

- New regulations for cell therapy in Taiwan and Singapore
- Approved gene therapy for cancer treatment in China
- Gene therapy regulatory practice in Japan
- Regenerative therapy in Japan
- Research in IPS

Dr Akihiro Shimosaka, Director, Research & Development, Research Foundation for Community Medicine

10.25 **Panel discussion**

11.00 Morning coffee in the exhibition area

FOLLOWED BY YOUR CHOICE OF 3 PARALLEL BREAKOUT SESSIONS:

FOCUS SESSION 1

Rigorous characterization of cell therapy products through the phases: Ensuring comparability

11.40 **Chair's introduction**

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

A series of case studies illustrating how comparability can be ensured as manufacturing scales up - Clarifying how the regulatory/GMP requirements are met at each stage: What constitutes a change (or not) in a product?

11.45 **Product vs process and implications for the commercialization of somatic cell therapies**

- Understanding the source of IP and industrial know-how in the context of a personalized somatic cell therapy
- Production challenges: Variability in starting material and effect on therapeutic benefit
- Regulatory issues in the context of rapid innovation

Gregg Sando, CEO, Cell Medica

12.05 Questions & discussion

12.10 **Phase I/II: Understanding the manufacturing process for allogeneic stem cells**

- Sourcing raw materials
- Generating new lines and cultures
- Ancillary materials
- Cryo-preservation
- Preparing to move to the next development stage

Dr Michael Schuster, Vice President, Operations, Angioblast Systems, Inc

12.30 Questions & discussion

12.35 **Transition from phase I/II to phase III: Manufacturing / quality decisions to support a pivotal trial and prepare for early stage commercialization**

- Vascugel clinical trial results
- Next steps for clinical advancement.
 - Trial design
 - Manufacturing- internal v. outsourced
- Quality systems decisions
 - Characterization and release assays
 - Assay development and validation
 - Increasing quality system robustness

- Process scale-up decisions
 - How and when
 - Comparability
 - Process validation

Jack Harvey MPH, Director, Manufacturing, Pervasis Therapeutics

12.55 Questions & discussion

1.00 Buffet lunch in the exhibition area

OR **Optional lunch workshop**
OR **Working lunch**

2.10 **Phase III: Aldagen's ALD-101**

- Facility scale-up and design
- When/why do you need to switch from one facility to another?
- What do you need to show to be able to switch from one facility to another?
- Multi-product manufacturing
- Protection from cross-contamination
- Chain of custody and identity
- Transport validation
- Product characterization

Ed Field, President & COO, Aldagen, Inc

2.30 Questions & discussion

2.35 **Phase III: Commercial scale CMC**

- Product characterization
- Process characterization
- Process validation
- Conformance lots and comparability
- Preparing for commercial launch

Michael Covington, Senior Director of Quality Assurance, Dendreon Corporation

2.55 Questions & discussion

3.00 **Case study & panel discussion**
Planning well ahead for regional / global manufacturing and scale-up: How to ensure consistency and comparability

- What is required to gain a license to manufacture at a specific facility?

Panelist:
Gary C. du Moulin, PhD, MPH, Senior Director, Quality Compliance, Genzyme Biosurgery

3.55 **Moderator's closing summary**

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

FOLLOWED BY AFTERNOON PLENARY SESSION

FOCUS SESSION 2

Preparing for combination therapies: Putting in place the regulatory, development and commercial pathways necessary to realize the potential of cell + gene + device + small molecule + biologic products

11.40 **Chair's introduction**

Dr Rajesh Chopra, Global Medical Science Director, Emerging Products, AstraZeneca

11.45 **What is the commercial potential of cell, gene and tissue therapies in combination with devices, with small molecules, and with each other?**

- Which disease areas will be most suited to their application
- Orthopaedic? • Cancer?
- Which combinations are most likely to improve delivery for cell and gene therapies?
- Matrixes and scaffolds? • Drugs? • Other biologics?
- What product life extension opportunities are emerging as a result of these combinations?

Speaker to be announced

12.05 Questions & discussion

12.10 **How is the regulator preparing for the complexity of assessing the various combinations of cell and gene therapies with other products?**

- What processes have been established to streamline regulatory oversight?
- What types of studies will be required for combinations of cell/gene therapies with drugs that are already on the market?
- How will the safety and quality of the matrix or scaffold be assessed when used in combination with cell/gene therapy?

Kimberly A. Benton, PhD, Deputy Director, Division of Cellular & Gene Therapies, OCTGT/CBER, US Food & Drug Administration

12.30 Questions & discussion

Case studies illustrating the decisions taken in selecting a particular combination, and how the development and regulatory hurdles are being overcome

12.35 **Experiences during phase I/II and how we are moving to marketing authorization during phase III**

- Scale-up Strategy for a gene therapy medicinal product

- Clarifying how GMP and regulatory requirements are achieved during phase I/II
- Process characterization and comparability
 - Release assays
 - Potency assays
 - Purity and functional assays
- Preparing to move to the next development stage

Marco Dieci BSc, Quality & Regulatory Director, Qualified Person, MolMed SpA

12.55 Questions & discussion

1.00 Buffet lunch in the exhibition area

OR **Optional lunch workshop**
OR **Working lunch**

2.10 **Urologic**

- How and when are the products combined?
- Specific issues when designing clinical trials
- How to dose?
- What's in the regulatory package? How do requirements between US and EU vary?

Elyse Seltzer, MD, Chief Medical Officer, Vice President, Clinical Development & Medical Affairs, Tengion, Inc

2.30 Questions & discussion

2.35 **Cardiac**

- Cell therapy for heart failure
 - Myoblasts, gene modified myoblasts, cell combinations
 - Dosing
 - Clinical trial design
- Cell therapy for myocardial infarction
 - Adipose tissue-derived stem cells
 - Dosing
 - Timing of delivery
 - Clinical trial design
- Repeated dosing of cell therapies
 - Myoblast experience in heart failure

Howard J. Leonhardt, Chairman, Chief Executive Officer & Chief Technology Officer, Bioheart, Inc

2.55 Questions & discussion

3.00 **Cardiac: Myocardial delivery of AAV1/SERCA2a in subjects with advanced heart failure - A first-in-human clinical trial**
Dr Krisztina Zsebo, CEO, Celladon Corporation (*pending final confirmation*)

3.20 Questions & discussion

3.25 **Panel discussion**

- **Is everyone doing enough to prepare for the growth of combination product opportunities?**
- How will levels of risk be assessed for combination products?

3.55 **Moderator's closing summary**

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

FOLLOWED BY AFTERNOON PLENARY SESSION

OR | WORKSHOP

The EU regulatory framework for Advanced Therapy Medicinal Products: Practical advice illustrating the realities of its implementation and the differences between member states' interpretation

Highly interactive workshop for a maximum of 30 participants

11.40 **Moderator's introduction**

Why these new regulations were needed in Europe

- Background to their development
- The 'difficult birth' of these regulations
- Implementation and current status

Dr Alan Boyd, Managing Director, alanboyd consultants Ltd

12.05 Questions & discussion

12.10 **Practical guide through precisely what to watch out for based on particular countries' interpretations and actions to date**

- Borderline products
- Regulatory gaps
- Implementation of Regulation 1394/2007
 - Formation of the CAT
 - Final version of Annex I
 - Definitions for cell therapy and gene therapy
 - Certification procedure
 - Traceability
- Interface between the EUCTD and Medicines Legislation
- EUTCD issues for ATMP's
 - Traceability
 - National differences in procurement and testing requirements under the EUCTD
 - How will these be handled within a centralized marketing authorization?
 - How will these affect clinical trials?

Dr Christopher Bravery, Director of Regulatory Affairs - Advanced Therapy Medicinal Products, ERA Consulting Group

12.30 Questions & discussion

12.35 **The UK regulator's view**

- MHRA's approach
- Scientific advice procedure
- Role as Rapporteur and as Non-Rapporteur
- Advisory committees and their role

- Borderline products and challenges
- Hospital exemption

Dr Gopalan Narayanan, Manager & Head, Biologicals & Biotechnology Unit, Medicines & Healthcare Products Regulatory Agency (MHRA)

12.55 Questions & discussion

1.00 Buffet lunch in the exhibition area

OR **Optional lunch workshop**
OR **Working lunch**

2.10 **Case study**

Building a European regulatory file for an ATMP: The ChondroCelect experience

- Meeting the different European regulatory requirements
- Manufacturing and releasing an autologous cell product
- Non-clinical evidence and documentation
- Running a clinical trial for cartilage repair
- Documenting the clinical safety and efficacy
- Learnings for future submissions of ATMP

Dr Wilfried Dalemans, Vice President, Regulatory Affairs & Corporate Quality, TiGenix NV

2.30 Questions & discussion

2.35 **Case study**

Taking it global: What you really need to know about study start-up

- The EU Directive provides regulations and guidelines for obtaining approvals necessary to begin a clinical study in the EU. However, the peculiarities and specifics are usually discovered long after timelines and budgets are set down. This presentation will discuss:
 - Requirements for specific member states that are not identified in the regulations and guidance's,
 - Member state specific issues pertaining to contracts and budgets and recommendations as to how to manage them,
 - Specific requirements pertaining to applications for approval of gene therapy products, and
 - Implications for sponsors who do not have a presence outside the United States.

Linda Strause, PhD, Executive Director, Oncology Clinical Operations, Vical Incorporated

2.55 Panel discussion

3.55 **Moderator's closing summary**

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

FOLLOWED BY AFTERNOON PLENARY SESSION

REGISTER BEFORE FRIDAY 31st OCTOBER TO SAVE \$200!

THEN AFTERNOON PLENARY SESSION

Ongoing opportunities for - and barriers to - cell, gene and tissue therapy

- Which diseases are now considered most amenable to their application? • How will they fare against the competition?

4.40 **Panel discussion OPPORTUNITIES:**

- What makes a good cell or gene therapy target?
- Developing a framework to enable the evaluation of indications and assess where strengths and weaknesses lie
- How do safety, benefit:risk, and time to market compare?

BARRIERS:

- Countering the negative impact of 'rogue' clinics and medical tourism: How can the opportunities and value be retained in the west?
 - What should clinicians be telling their patients about the value of these therapies?

- Harvesting the human clinical data that is going to waste
- How will cell, gene and tissue therapies fare against competition from cocktails of small molecules and growth factors?

Panelists:

Paul J. Schmitt, Managing Director, Novitas Capital
Shosh Merchav, PhD, MBA, Director, Head, Cell Therapy Projects, Teva Innovative Venture, Teva Pharmaceutical Industries Ltd
Dr Geert-Jan Mulder, General Partner, Forbion Capital Partners
Anthony G. Coia, MD, Principal, BioVentures Investors

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

7.30 Registration & buffet breakfast in the exhibition area

MORNING PLENARY SESSION

Gaining an insight into big pharma and big biotech's current and future plans for cell, gene and tissue regeneration therapies
- Analyzing internal and external investment, and collaborative moves

9.00 Chair's introduction

Edward Lanphier, President & CEO, Sangamo BioSciences, Inc

Recent case studies of companies buying, investing in, and partnering with cell and gene therapy, and tissue engineering companies

9.05 Genzyme/Shanghai Sunway Biotech deal: How will it speed the pathway for getting gene therapy into global markets?

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

9.25 Questions & discussion

9.30 GlaxoSmithKline and the Harvard Stem Cell Institute: An example of collaboration between pharma and academia in early stage science

Brook C. Reeve, Executive Director, Harvard Stem Cell Institute

9.50 Questions & discussion

9.55 Panel discussion

How is big pharma/big biotech's interest in the cell, gene and tissue regeneration sector now manifesting itself?

- What is our ongoing strategy for involvement in the West and the East?
- How are companies ramping up their internal capabilities in the cell, gene and tissue regeneration sectors?
- How are Asian subsidiaries approaching these new therapies?
- What does the product profile need to look like to turn them into fundable technologies?

Panellists:

Dr Ajan Reginald, Global Head of Emerging Technologies, Roche Group Research
Dr Rajesh Chopra, Global Medical Science Director, Emerging Products, AstraZeneca
Dr Sudha Kadiyala, Worldwide Director, Business Development & Strategic Planning, Johnson & Johnson
Shosh Merchav, PhD, MBA, Director, Head, Cell Therapy Projects, Teva Innovative Venture, Teva Pharmaceutical Industries Ltd
Dr John McNeish, Executive Director, Regenerative Medicine, Pfizer

11.20 Morning coffee in the exhibition area

FOLLOWED BY YOUR CHOICE OF 3 PARALLEL BREAKOUT SESSIONS:

FOCUS SESSION 1

Viral and non-viral vectors for the targeted delivery of gene-based therapies: Advances in safety, regulation, manufacturing and scale-up

12.00 Chair's introduction

Dr Karen Kozarsky, Head, Gene Therapy, Biopharmaceutical Center of Excellence for Drug Discovery, GlaxoSmithKline

12.05 Preclinical considerations for gene therapy products: CBER perspective

- Considerations in designing proof-of-concept studies
- Considerations in designing toxicology/bio-distribution studies

Wei Liang, PhD, Pharmacologist, Division of Clinical Evaluation & Pharmacology/Toxicology, OCTGT/CBER, US Food & Drug Administration

12.25 Questions & discussion

Case studies

Viral and non-viral delivery mechanisms in clinical trials: Integrating each specialized development function into one cohesive, synchronized plan

- Toxicity
- Specificity
- Durability
- Host responses
- Re-administration/immunogenicity issues
- Regulatory interaction
- Manufacturing hurdles
- Timelines for commercial adoption
- Planning worldwide product launch

12.30 AAV vectors

- Toxicity and host response
- Ability to readminister
- CMC challenges
- Ease of manufacture
- Regulatory interactions
- Clinical development strategy

Professor Sander van Deventer, CSO, AMT BV

12.50 Questions & discussion

12.55 Buffet lunch in the exhibition area

2.05 Lentiviral vectors

- CMC challenges for novel gene therapy platforms
 - Vector
 - Manufacture
 - 'Appropriate' release assays
- Non-clinical development
 - Design of appropriate toxicology packages for long term gene-based therapy
- Regulatory interactions
- Moving to the clinic
 - Study design
 - Clinical supplies
 - Clinical sites
- Progressing from a First in Man study
- Manufacturing for phase III and beyond
- Clinical development strategy
- Pricing 'one shot' treatment

Dr Stuart Naylor, Chief Scientific Officer, Oxford Biomedica (UK) Ltd

2.25 Questions & discussion

2.30 Non-viral gene therapy: DNA nanoparticles

- Lack of toxicity and host response
- Ability to readminister
- Long-term expression
- Ease of manufacture
- Favorable regulatory climate for non-viral approach
- Clinical applications

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

2.50 Questions & discussion

2.55 Making drugs out of siRNAs

- Mechanism of RNA interference
- Role of chemical modifications and formulations
- Addressing off-target effects
- Local and systemic delivery approaches
- Ongoing clinical trials

Muthiah Manoharan PhD, Vice President, Drug Discovery, Alnylam Pharmaceuticals

3.15 Questions & discussion

3.20 RNA therapy roundtable

What promising technologies are coming out of research and how are they progressing in the clinic?

- How will different therapeutic options be regulated and how will this differ from 'standard' gene therapies?
- How will this impact their development path and progress?
- What advances are being made in specificity and delivery of RNA therapies?
 - Antisense
 - siRNA
 - miRNA
 - shRNA

Panellist:
Henrik Ørum, MSc, PhD, Vice President & CSO, Santaris Pharma A/S

3.55 Moderator's closing summary

4.00 Close of the Cell & Gene Therapy Forum 2009 followed by afternoon tea in the exhibition area

OR | FOCUS SESSION 2

Assessing the therapeutic performance of stem cells from different sources

- How is a better understanding of mechanism of action impacting their application to particular disease environments?

12.00 Chair's introduction

Alternative sources of stem cells – natural and synthetic: Which have real potential?

- Which are ethically acceptable?

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

Case studies

Taking three different sources of stem cells to examine the capabilities and benefits of each

- How thorough is the understanding of the mechanism of action?
- What are the specific toxicity and safety issues for each?
- How do you determine the potency of a stem cell?
- What might the regulatory pathway look like?
- How would they fare in the clinic?
- Can they yield enough to be cost effective?
- What are the manufacturing and scale-up issues?

12.05 Development of hESC derived cell therapy for diabetes

- Pros and cons of hESC technology and directed differentiation
- Why diabetes cell therapy?
- Progenitor cell products versus differentiated cell products
- Regulatory pathway for hESC derived cell product: Characterization, efficacy and safety
- Is immunosuppression or immunomodulation the preferred procedure to protect against transplant rejection?

Dr Alan J. Lewis, President & CEO, Novocell Inc

12.25 Questions & discussion

12.30 Induced pluripotent stem cells

- Would they be considered genetically modified?
- What do these genetic differences mean for industry?
- Is it a gene or a cell therapy?
- How will they be classified and how will they be regulated?

John T. Dimos, PhD, Senior Scientist, iZumi Bio Inc

12.50 Questions & discussion

12.55 Buffet lunch in the exhibition area

2.05 Mesenchymal stem cells

Osiris Therapeutics: Commercializing stem cell therapies

- Mesenchymal stem cell (MSC) technology
- GMP manufacturing of MSCs
- Clinical progress: Where we're going and where we've been

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

2.25 Questions & discussion

2.30 Immunogenicity of stem cells: Industry experiences of what the regulator might be looking for

- What types of assays are required?
- Correlating immunogenicity of cells with your therapeutic results
- Clarifying how the tests are being interpreted

Bruce Wentworth, PhD, Senior Director – Science, MG Biotherapeutics & Genzyme

2.50 Questions & discussion

2.55 Directed differentiation of stem cells: Why is it important and how to achieve it?

- Factors affecting efficient differentiation
- Patent thickets: Will there be trouble later?
- How to achieve cost-effective and reproducible protocols
- High throughput screening methods for optimal directed differentiation

Dr Yen Choo, Founder & CEO, Plasticell

3.15 Questions & discussion

3.20 Panel discussion

Which cell type is the right one to use?

- What questions should companies ask in order to select one cell type over another and minimize investment risk?
 - What other sources of stem cells are being evaluated and with what results?
 - How can cell companies prove that theirs is better?
 - There are too many unknowns – what are we doing to address this?

3.55 Moderator's closing summary

4.00 Close of the Cell & Gene Therapy Forum 2009 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 for more information.



OR | WORKSHOP

Financing global growth in the cell, gene and tissue therapy sector

- What sources of funding are now being exploited?
- Are investors evaluating these opportunities appropriately?

Highly interactive workshop for a maximum of 30 participants

12.00 What are the alternatives to VC funding?

- Summary of recent major financing events
- What impact is the increasing interest from big pharma having on the funding environment?

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

Case studies demonstrating the application of different sources of funding, how the companies attracted the capital, and how the investors evaluated the risk:reward

- Partnerships with research tools companies
- Corporate venture financing
- Foundations / philanthropic investors
- Government money
- Equity funding – IPOs

12.05 Employing a hybrid business and financing model to develop novel ZPF therapeutics

- Intellectual property: Required for raising capital
- Funding alternatives: Venture, public markets, grants, foundations
- Partnerships in core and non-core markets
- Cash and dilution management

Edward Lanphier, President & CEO, Sangamo BioSciences, Inc

12.25 Questions & discussion

12.30 Surrounded by insurmountable opportunities: Using creative financing strategies to move science ahead

- Use of private/public partnerships as financing vehicles
- Collaborative financing strategies
- Novel strategies to compensate for faltering markets or lack of pharma early stage interest

H. Stewart Parker, President & CEO, Targeted Genetics Corporation

12.50 Questions & discussion

12.55 Buffet lunch in the exhibition area

2.05 NIH funding for translational science: Public private partnerships in the CTSA environment (Clinical and Translational Science Award)

Dr Mark Scheideler, Senior Scientific Officer, Roadmap Initiatives, National Institute of Neurological Disorders & Stroke, National Institutes of Health

2.25 Questions & discussion

2.30 Best practice due diligence for the cell and gene therapy sector: What criteria should investors be evaluating when making investment decisions, and how can companies better prepare for this?

- IP due diligence

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

2.50 Questions & discussion

2.55 Panel discussion

VC response: What do we see as core evaluation criteria for the cell and gene therapy sector?

- To what extent does the product vs service debate apply to cell and gene therapies, and how does it influence financing options and investment cycles?

Panellists:

Paul J. Schmitt, Managing Director, Novitas Capital
Gregory A. Bonfiglio, Managing Partner, Proteus Venture Partners
Dr Geert-Jan Mulder, General Partner, Forblon Capital Partners
Anthony G. Coia, MD, Principal, BioVentures Investors

3.55 Moderator's closing summary

4.00 Close of the Cell & Gene Therapy Forum 2009 followed by afternoon tea in the exhibition area

Phacilitate calendar of events

Phacilitate Vaccine Forum Washington 2009

Running concurrently with the Cell & Gene Therapy Forum - shared networking!

26-28 January,
The Grand Hyatt
Washington, DC
www.phacilitate.co.uk/wv

Phacilitate Vaccine Forum Barcelona 2009

22-24 June,
The Fira Palace
Barcelona
www.phacilitate.co.uk/barcelona

Phacilitate Active Immunotherapeutics Forum 2009

22-24 June,
The Fira Palace
Barcelona
www.phacilitate.co.uk/barcelona

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Sponsorship and exhibition opportunities

The combined Cell & Gene Therapy Forum 2009 and Washington Vaccine Forum 2009 provides an unparalleled networking and profile-raising opportunity to companies targeting these sectors. All networking activities are shared in a central exhibit area, giving you a great chance to meet with numerous senior level R&D, regulatory, business development, manufacturing executives all under one roof and in just three days!

"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"

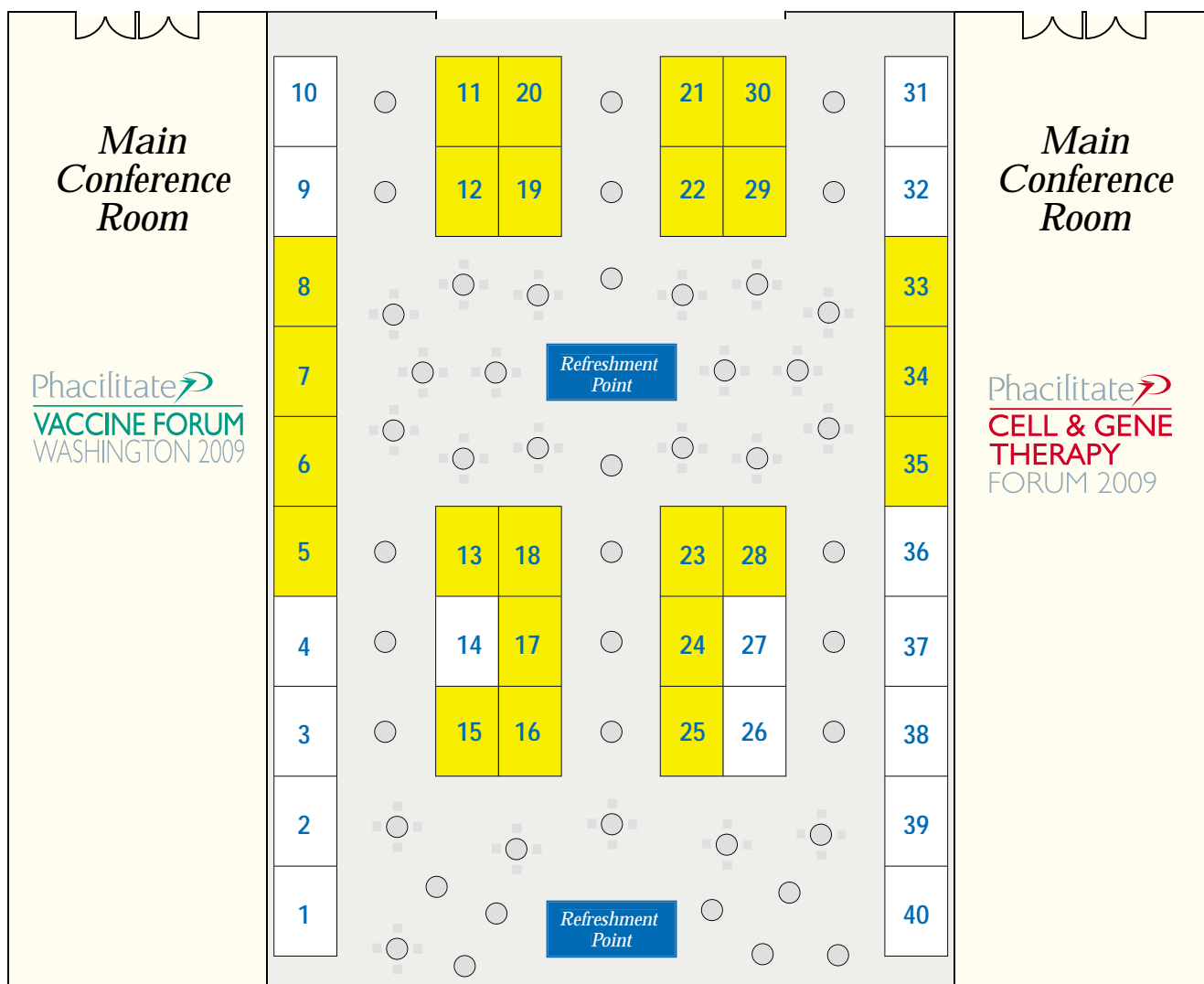
Megan Barth, Director of Sales, Pharmaceutical & Biotechnology, Sterigenics

"The Cell and Gene Therapy Forum / Vaccine Forum held in January 2008 was a very successful event for the ERA Consulting Group. Not only was this a unique opportunity for us to have meetings with many of our current clients, but we also successfully secured 4 new clients at this event. ERA will certainly be attending the 2009 event."

Paul Cronin, Business Development Manger, ERA Consulting Group

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 2009 and the Washington Vaccine Forum 2009 - over 500 senior life science executives with the authority to impact your business!



Exhibitors:

- Booth 5 Eden Biodesign
- Booth 6 WuXi AppTec
- Booth 7 Bridge Laboratories
- Booth 8 Progenitor Cell Therapy
- Booth 11 Accelovance
- Booth 12 MGlas
- Booth 13 Worthington Biochemical Corporation
- Booth 15 Angel Biotechnology PLC
- Booth 16 GE Healthcare
- Booth 17 BD Medical - Pharmaceutical Systems
- Booth 18 Pall Life Sciences
- Booth 19 Cobra Biomanufacturing
- Booth 20 DynPort Vaccine Company LLC, A CSC Company

- Booth 21 Beardsworth Consulting Group
- Booth 22 Covance Research Products
- Booth 23 PRTM
- Booth 24 Aldevron
- Booth 25 BioLife Solutions
- Booth 28 Novozymes Biopharma
- Booth 29 Eufets
- Booth 30 Diamyd Medical
- Booth 33 ERA Consulting
- Booth 34 Lonza
- Booth 35 Ichor Medical Systems
- Plus table-top exhibitors
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- Promoting research, development and clinical application of gene therapies
- Exchanging information and promoting education among professionals and the public about gene therapy, and
- Promoting development of clinical translations of gene therapy for a variety of diseases

www.asgt.org

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Further information is available at: www.bsgt.org

The European Society of Gene & Cell Therapy

The primary objectives of the European Society of Gene and Cell Therapy (ESGCT), formerly called the European Society of Gene Therapy (ESGT), are to promote basic and clinical research in gene therapy, cell therapy, and genetic vaccines to promote education and the exchange of information and technology related to above areas and to serve as a professional adviser to the community and to the regulatory bodies in Europe.

The ESGCT annual congress provides a platform for promoting basic and clinical research by facilitating education and by sharing of information and technology. The ESGCT Congress 2008 will be held in the beautiful town of Brugge, Belgium.

