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Dr Nick Crabb,
Programme Director, Scientific Affairs, Centre for Health Technology Evaluation,
National Institute for Health & Care Excellence

Dr Christiane Niederlaender,
CAT Member; Senior Quality Assessor,
UK Medicines and Healthcare Products Regulatory Agency (MHRA)

Dr Joseph Rabinowitz,
Senior Director of AAV Capsid Development,
Pfizer

Dr Hans Middelhoven,
Head Innovative Pricing, Global Pricing and Market Access,
F-Hoffman - La Roche

Dr Mark Lowdell,
Director of Cellular Therapeutics, **Royal Free Hospital;** *Professor of Cell & Tissue Therapy,* **University College London**

Andrea Spezzi, MD, FFPM,
Chief Medical Officer & Head of R&D
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Dr Christiane Niederlaender, *CAT Member; Senior Quality Assessor,* **UK Medicines and Healthcare Products Regulatory Agency (MHRA)**

Eric Falcand, *Vice President, Business Development & Licensing,* **Servier**

Angela Crossman, *Global Market Access Director, Gene Therapy,* **GlaxoSmithKline**

Dr Romaldas Maciulaitis, *Clinical & Regulatory Expert; EMA CAT & CHMP Member,* **Lithuanian State Medicines Control Agency**

Rocio Salvador-Roldan, *Policy Officer, DG Health,* **European Commission**

Dr David Gilham, *Vice President R&D,* **Celyad**

Dr Hans Middelhoven, *Head Innovative Pricing, Global Pricing and Market Access,* **F-Hoffman- La Roche**

Dr Detlev Parow, *Head of Department, Care Management Development,* **DAK-Gesundheit – Germany**

Dr Joseph Rabinowitz, *Senior Director of AAV Capsid Development,* **Pfizer**

Dr Lothar Germeroth, *Senior Vice President,* **Juno Therapeutics, Inc;** *Managing Director,* **Juno Therapeutics GmbH**

Dr Jim Faulkner, *Senior Vice President & Head of Manufacturing,* **Autolus Ltd**

Dr Stefan Gluck, *Vice President, Global Medical Affairs, Immunotherapy,* **Celgene**

Dr Nick Crabb, *Programme Director, Scientific Affairs, Centre for Health Technology Evaluation,* **National Institute for Health & Care Excellence**

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Dr Dmitry Kuzmin, *Managing Partner,* **4BIO Capital**

Stéphan Reynier, *Chief Regulatory & Compliance Officer,* **Cellectis**

Dr Diego Ardigo, *Project Leader, ATMP's,* **Chiesi Farmaceutici SpA**

Prakash Raman, *Vice President & Global Head of Oncology BD&L,* **Novartis**

Dr Steven Howe, *Head, Process Research, Cell & Gene Therapy Platform CMC,* **GlaxoSmithKline**

Dr Mark Lowdell, *Director of Cellular Therapeutics, Royal Free Hospital; Professor of Cell & Tissue Therapy,* **University College London**

Frédéric Revah, PhD, *President,* **YposKesi;** *CEO,* **Genethon**

Dr Jan Thirkettle, *CEO & Chief Development Officer,* **Freeline Therapeutics**

Andrea Spezzi, MD, FFPM, *Chief Medical Officer & Head of R&D,* **Orchard Therapeutics**

Dr Dariusz Śladowski, *Member, Committee for Advanced Therapies (CAT),* **European Medicines Agency (EMA)**

Dr Fraser Wright, *Co-Founder & Chief Technology Officer,* **Spark Therapeutics** *(provisionally confirmed)*

Dr Eugenio Montini, *Head of Unit, Safety of Gene Therapy & Insertional Mutagenesis Research Unit,* **San Raffaele Telethon Institute for Gene Therapy (SR-TIGET)**

Dr Robert Deans, *CTO,* **BlueRock Therapeutics**

Dr Benoît Champluvier, *Chief Technology & Manufacturing Officer,* **Bone Therapeutics**

Dr Helen Tayton-Martin, *Chief Business Officer,* **Adaptimmune**

Dr Christopher Bravery, *Director,* **Advanced Biologicals Ltd**

Dr Thierry Laugel, *Managing Partner,* **Kurma Partners**

Ajan Reginald, *CEO,* **Celixir**

Dr Giuliana Vallanti, *Development & Quality Control Director, Qualified Person,* **MolMed**

Dr Sandra van Wetering, *COO,* **DCPrime BV**

Dr Niranjan Sardesai, *Chief Operating Officer,* **Inovio Pharmaceuticals, Inc**

Dr Anthony Davies, *President,* **Dark Horse Consulting**

Wilfried Dalemans, *Chief Technical Officer,* **TiGenix**

Matthew Durdy, *Chief Business Officer,* **Cell and Gene Therapy Catapult**

Sharon Grimster, *General Manager, Wales,* **ReNeuron**

Phil Vanek, *General Manager, Cell Bioprocessing,* **GE Healthcare**

Dr Giovanni Mariggi, *Principal,* **Medicxi**

Eric Dutang, *CFO,* **Cellectis**

Alexander Scheer, PhD, *Chief Scientific Officer,* **Erytech**

Dr Madhusudan Peshwa, *Chief Scientific Officer, Executive Vice President, Cellular Therapies,* **MaxCyte, Inc**

John Lunger, *Vice President, Manufacturing & Supply Chain,* **Adaptimmune**

Dr Kai Pinkernell, *Senior Vice President, Medical Affairs & CMO,* **Medigene AG**

Dr Cedrik Britten, *Head, Cellular Therapy- Immuno-Oncology,* **GlaxoSmithKline**

Dr Knut Niss, *Vice President, Program Management,* **Mustang Bio**

Anne-Virginie Eggimann, *Vice President, Regulatory Science,* **bluebird bio, Inc**

Professor Miguel Forte, *Chief Medical Officer,* **Bone Therapeutics;** *Managing Director,* **mC4Tx** *(modified Cell for Therapeutics); Chief Commercialisation Officer and Chair of Commercialisation Committee,* **ISCT**

Sharron Gargosky, PhD, *Chief Clinical & Regulatory Officer,* **OncoSec Medical Incorporated**

Dr Janneke Meulenberg, *COO,* **Arthrogen**

Dr Maya Fürstenau-Sharp, *Field Marketing Manager EU, Regenerative Medicine,* **Sartorius Stedim Biotech GmbH**

Patrick Jeanmart, *CFO,* **Celyad**

Dr Hicham Majd, *Device Manager - Device Technology, Biologics Technical Development and Manufacturing (BTDM),* **Novartis Pharma AG**

Dr Luca Alberici, *Director of Business Development,* **MolMed**

Dr Toby Toward, *Head of Commercial Strategy,* **Immunocore**

Dr Cenk Sumen, *Senior Manager, Business Development,* **PCT**

Dr Matthew Lakelin, *Vice President, Scientific Affairs,* **TrakCel**

Boro Dropulic, PhD, MBA, *Chief Scientific Officer & General Manager,* **Lentigen Technology, Inc A Miltenyi Company**

Sabine Schöffel-Weiß, *Deputy Director,* **CMS Cellex Medical Services GmbH**

Seokjoong Kim, PhD, *Research Director,* **ToolGen, Inc**

Dr Christine Guenther, *CEO & Medical Director,* **apceth Biopharma GmbH**

Dr Bernd Leistler, *Vice President Development & Production,* **CellGenix**

Dr Christina Basford, *Manager,* **GlaxoSmithKline**

Dr Lincoln Tsang, *Partner, Life Science Practice,* **Arnold & Porter Kaye Scholer LLP**

Dr Richard Janeczko, *Chief Executive Officer,* **VEL Vaccines Biosciences Inc**

Dr David Venables, *CEO,* **Synpromics**

Dr Gopalan Narayanan, *Vice President, Disruptive Biologics,* **Voisin Consulting Life Sciences (VCLS)**

Dr Klaus Kühlcke, *Managing Director,* **EUFETS GmbH**

Dr Eric Soller, *Vice President of Corporate Development & Strategy,* **BlueRock Therapeutics**

Jan Spanholtz, PhD, *Chief Scientific Officer,* **Glycostem**

Dr Nicole Faust, *Chief Scientific Officer,* **CEVEC Pharmaceuticals**

Dr Damian Marshall, *Head of Analytical Development,* **Cell and Gene Therapy Catapult**

Dr Andres Gutierrez, *Chief Medical Officer,* **Oncolytics Biotech Inc**

Dr Tim Farries, *Director, Regulatory Affairs, Cell & Gene Therapies,* **ERA Consulting**

Professor Dr Mohamed Abou-El-Enein, *Head of Clinical Development Platform, Translational Research Specialist, Berlin-Brandenburger Centre for Regenerative Therapies (BCRT);* **Charité Universitätsmedizin Berlin**

Dr Stefano Baila, *Industrialization Manager,* **Celyad**

Jerrod Denham, *Principal & Senior Consultant,* **Dark Horse Consulting, Inc**

Frank Hecht, *Vice President Marketing & Sales,* **CellGenix**

Dr Christophe Queva, *CSO,* **iTeos Therapeutics**

Dr Nicolas Poirier, *CSO,* **OSE Immunotherapeutics**

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AGENDA

PRE-CONFERENCE TRAINING: TUESDAY, SEPTEMBER 19th 2017

9.00am-5.30pm A range of deep-dive ½-day Training sessions on the following topics:

Morning

Product characterisation/ Quality by Design

OR

Clinical trial design for ATMPs

Quality by Design (QbD) is a science- and risk-based approach to pharmaceutical development. Applied to manufacturing processes, it is intended to ensure a rational and cost- effective approach to product quality. Both the EMA and FDA have systematically assessed QbD elements of regulatory filings and, especially since the FDA's 2011 Process Validation Guidance and the ICH's Q8 through Q11, it has become an intrinsic part of regulatory guidance.

However, understanding and adoption of the principles of QbD has remained relatively limited in the field of cell and gene therapy. We strongly believe that its methodologies are critical to the successful development of ATMPs and that they should be incorporated as early as possible into the science of manufacturing. This workshop will both demystify the fundamentals of QbD and provide concrete examples of its application to this field. You will leave with a crystal-clear understanding of P Diagrams, RAMM matrices, the QTPP and many more tools which you can immediately use to improve your products and processes.

Led by:

Dr Anthony Davies, *President,* **Dark Horse Consulting**

Jerrod Denham, *Principal & Senior Consultant,* **Dark Horse Consulting, Inc**

The considerations when initiating a clinical trial for cell-based therapies are diverse and numerous. Alternative approaches to traditional clinical development based on sequential, seamless design are justified, including exploratory (phase I/IIa) trials examining both safety and efficacy while using an extensive biomarker portfolio, whenever applicable. Great emphasis should nevertheless be placed on patient safety, particularly in designing appropriate GMP- grade product and risk-benefit related stopping criteria. In this session some of these aspects will be discussed using real-life examples.

In this session, Professor Dr Abou-El Anein will be joined by his colleague, Mr Daniel Kaiser who will discuss the characteristics of a clinical grade product to be used in clinical trials.

Led by:

Professor Dr Mohamed Abou-El-Enein, *Head of Clinical Development Platform, Translational Research Specialist, Berlin-Brandenburger Centre for Regenerative Therapies (BCRT);* **Charité Universitätsmedizin Berlin**

Lunch break

Authoring the Common Technical Document (CTD) Module 3 (CMC/Quality)

The ICH Common Technical Document was developed to standardise the way information is shared with regulators, and is accepted or mandatory by agencies following ICH (e.g. EU, US, Japan, Canada etc). The CTD is used for all types of medicinal product but the purpose of each section may not always be clear for ATMP. This workshop aims to clarify:

- What the purpose of each section is
- Whether the section is descriptive or data-driven
- What sorts of data are expected
- Tips and considerations for style, use of figures and tables, etc.

Led by:

Dr Christopher Bravery, *Director,* **Advanced Biologicals Ltd**

...followed by an interactive site visit to the GMP facility at the Charité Hospital in Berlin, which is open to all attendees.

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Pre-conference Training Day

Offering expert led, deep-dive regulatory workshops tailored to the needs of a globalized industry, available to delegates for just an extra £500 + VAT (non delegates can attend for £750 + VAT)

AGENDA

DAY 1: WEDNESDAY, SEPTEMBER 20th 2017

7.15 REGISTRATION & BREAKFAST IN THE EXHIBITION AREA

Opening morning plenary

HOW TO ENSURE ATMPS CONTINUE TO FLOURISH AS A KEY GROWTH DRIVER ACROSS EUROPE?

8.45 CHAIR'S INTRODUCTION
Dr Anthony Davies, *President,* **Dark Horse Consulting**

8.50 INTRODUCTORY OVERVIEW: REVIEWING SIGNIFICANT RECENT AND EXPECTED FORTHCOMING EVENTS IN THE ATMP SPACE WORLDWIDE – WHAT DO THEY TELL US ABOUT THE RELATIVE STRENGTHS AND WEAKNESSES OF THE EUROPEAN SECTOR?
Dr Mark Lowdell, *Director of Cellular Therapeutics, Royal Free Hospital; Professor of Cell & Tissue Therapy,* **University College London**

9.10 INVESTOR ROUNDTABLE
Dr Dmitry Kuzmin, *Managing Partner,* **4BIO Capital**
Dr Thierry Laugel, *Managing Partner,* **Kurma Partners**
Dr Giovanni Mariggi, *Principal,* **Medicxi**
Dr Eric Soller, *Vice President of Corporate Development & Strategy,* **BlueRock Therapeutics;** *formerly Entrepreneur in Residence,* **Versant Ventures**

9.40 EXECUTIVE SUMMARY OF EUROPEAN REGULATORY EVOLUTION
Rocio Salvador-Roldan, *Policy Officer, DG Health,* **European Commission**
Dr Lincoln Tsang, *Partner, Life Science Practice,* **Arnold & Porter Kaye Scholer LLP**

10.05 PRESENTATION RESERVED

10.25 CLOSE OF SESSION

10.30-11.15

Phacilitate Lab Sessions

Your choice of 40-minute sessions in a variety of highly interactive formats, including: Various small roundtable discussions on specific manufacturing, regulatory and translational R&D themes

RAW MATERIALS FOR ATMP MANUFACTURING: REGULATORY AND SUPPLY ISSUES
Moderators:
Dr Bernd Leistler, *Vice President Development & Production,* **CellGenix**
Frank Hecht, *Vice President Marketing & Sales,* **CellGenix**

A BIG PHARMA R&D PORTFOLIO/LICENSING & PARTNERING STRATEGY Q&A
Panellists:
Prakash Raman, *Vice President & Global Head of Oncology BD&L,* **Novartis**
Dr Sven Kili, *Vice President & Head of Cell & Gene Therapy Development, Rare Disease Unit,* **GlaxoSmithKline**

11.15 CLOSE OF SESSION – MORNING COFFEE IN THE EXHIBITION AREA

Followed by your choice of highly interactive breakout tracks:

Track 1: Cell-based therapy business & innovation

11.45-1.30pm

COST EFFECTIVE SCALE UP AND OUT MODELS TO DRIVE THE COMMERCIAL FEASIBILITY OF ATMPS IN EUROPE – PART 1

- Comparing and contrasting the differing scale up and scale out strategies of ATMP industry leaders
- Important design considerations for the development of a cGMP compliant manufacturing process for autologous cell therapy applications
- To centralise or decentralise? Examining the full spectrum of potential models available for ATMP manufacturing, spanning remote facility to point of care – how realistic is each option for commercial scale production in Europe over the relatively near term?

Chair: Phil Vanek, *General Manager, Cell Bioprocessing,* **GE Healthcare**
Dr Alexander Huber, *Global CMC Head & Director, Cell & Gene Therapy Unit,* **Novartis Pharma AG**
Dr Lothar Germeroth, *Senior Vice President,* **Juno Therapeutics, Inc;** *Managing Director,* **Juno Therapeutics GmbH**
Dr Behnam Ahmadian Baghbaderani, *Head of Cell Therapy Development,* **Lonza**
Stewart Craig, PhD, *Chief Manufacturing Officer,* **Orchard Therapeutics**
Dr Kai Pinkernell, *Senior Vice President, Medical Affairs & CMO,* **Medigene AG**
Dr Benoît Champluvier, *Chief Technology & Manufacturing Officer,* **Bone Therapeutics**

Track 2: R&D updates & emerging science

11.45-1.30pm

ENGINEERED STEM CELLS: THE NEXT GREAT INNOVATION WAVE IN CELL & GENE THERAPY?

- Case studies: Does recent data make the case for genetically-modified stem cells supplanting cellular immunotherapies as tomorrow's hot tech area in the ATMP field? Examining the latest approaches – to what extent are they:
- Addressing long-standing safety and other regulatory concerns surrounding stem cell-based products (eg. carcinogenesis, inappropriate differentiation)?
- Suggesting they can deliver the sort of game-changing efficacy the field has been waiting for?
- Delivering previously missing insights into Mechanism of Action

Chair: Professor Miguel Forte, *Chief Medical Officer,* **Bone Therapeutics;** *Managing Director,* **mC4Tx (modified Cell for Therapeutics);** *Chief Commercialisation Officer and Chair of Commercialisation Committee,* **ISCT**
Andrea Spezzi, MD, FFPM, *Chief Medical Officer & Head of R&D,* **Orchard Therapeutics**
Dr Christine Guenther, *CEO & Medical Director,* **apceth Biopharma GmbH**
Dr Eugenio Montini, *Head of Unit, Safety of Gene Therapy & Insertional Mutagenesis Research Unit,* **San Raffaele Telethon Institute for Gene Therapy (SR-TIGET)**

Track 3: Vector business & innovation

11.45-1.30pm

WEIGHING UP IN-HOUSE AND OUTSOURCED VECTOR PRODUCTION STRATEGIES TO BEAT EUROPE'S CAPACITY BOTTLENECK

- Framing the problem: quantifying the ATMP sector's current and future demand for AAV, lentiviral and retroviral vectors and mapping it to current/planned capacities

THEN

UPSCALING AND OPTIMISING VIRAL VECTOR PRODUCTION PLATFORMS TO MEET FUTURE ATMP INDUSTRY DEMAND: PART 2 – AAV

- Comparing and contrasting the scalability, cost effectiveness and production yield/quality of next-generation AAV vector production platforms – which ones are gearing up to meet future commercial scale demand?

Frédéric Revah, PhD, *President,* **YposKesi;** *CEO,* **Genethon**
Dr Joseph Rabinowitz, *Senior Director of AAV Capsid Development,* **Pfizer**
Dr Fraser Wright, *Co-Founder & Chief Technology Officer,* **Spark Therapeutics** *(provisionally confirmed)*
Boro Dropulic, PhD, MBA, *Chief Scientific Officer & General Manager,* **Lentigen Technology, Inc A Miltenyi Company**
Dr Janneke Meulenberg, *COO,* **Arthrogen**

Workshop Track
(for a maximum of 30 participants)

11.45-1.30pm

WHAT ARE THE NEED-TO-KNOW CHARACTERISTICS OF PRECLINICAL R&D SPECIFIC TO THE CELL, GENE AND IMMUNOTHERAPY FIELD?

- A roadmap to optimal clinical translational for novel biotherapeutic products in Europe
- Experiences from scientific discussions with EU regulators

Dr Romaldas Maciulaitis, *Clinical & Regulatory Expert; EMA CAT & CHMP Member,* **Lithuanian State Medicines Control Agency**

Dr Dariusz Sladowski, *Member, Committee for Advanced Therapies (CAT),* **European Medicines Agency (EMA)**

1.30 **Buffet lunch in the exhibition area**

AGENDA

Track 1: Cell-based therapy business & innovation

2.30-4.15pm

COST EFFECTIVE SCALE UP AND OUT MODELS TO DRIVE THE COMMERCIAL FEASIBILITY OF ATMPS IN EUROPE – PART 2

- Deriving competitive advantage from soaring demand for European cell-based therapy bioprocessing capacity
- How to ensure bioprocess development and optimisation stays in step with expedited/accelerated clinical development pathways?
- Understanding the full impact of bioprocess changes mid-clinical trial
- What are the critical considerations and learnings in terms of cell line development and optimisation in Europe with global development/commercialisation as the end game?

Chair: Dr Knut Niss, *Vice President, Program Management,* **Mustang Bio**
Dr Jim Faulkner, *Senior Vice President & Head of Manufacturing,* **Autolus Ltd**
Dr Christine Guenther, *CEO & Medical Director,* **apceth Biopharma GmbH**
Professor Miguel Forte, *Chief Medical Officer,* **Bone Therapeutics;** *Managing Director,* **mC4Tx** *(modified Cell for Therapeutics); Chief Commercialisation Officer and Chair of Commercialisation Committee,* **ISCT**
Dr Liesbeth de Jong, *Vice President, Manufacturing and Research & Development,* **Rexgenero**
Dr Christina Basford, *Manager,* **GlaxoSmithKline**

Track 2: R&D updates & emerging science

2.30-4.15pm

IMMUNO-ONCOLOGY: NOVEL TARGETS AND INNOVATIVE WAYS TO INDUCE A BETTER IMMUNE RESPONSE

- Case studies: Clinical innovation in immuno-oncology - what is coming through the clinical translational phase as the next wave of cancer therapeutics?

Dr Stefan Gluck, *Vice President, Global Medical Affairs, Immunotherapy,* **Celgene**

Stéphan Reynier, *Chief Regulatory & Compliance Officer,* **Cellectis**

Dr Christophe Queva, *CSO,* **iTeos Therapeutics**

Dr Nicolas Poirier, *CSO,* **OSE Immunotherapeutics**

Track 3: Vector business & innovation

2.30-4.15pm

UPSCALING AND OPTIMISING VIRAL VECTOR PRODUCTION PLATFORMS TO MEET FUTURE ATMP INDUSTRY DEMAND: PART 1 – LENTIVIRAL AND RETROVIRAL

- Comparing and contrasting the scalability, cost effectiveness and production yield/quality of next-generation lenti and retroviral vector production platforms – which ones are gearing up to meet future commercial scale demand?

Chair: Boro Dropulic, PhD, MBA, *Chief Scientific Officer & General Manager,* **Lentigen Technology, Inc A Miltenyi Company**

Dr Steven Howe, *Head, Process Research, Cell & Gene Therapy Platform CMC,* **GlaxoSmithKline**

Dr Klaus Kühlcke, *Managing Director,* **EUFETS GmbH**

Dr Nicole Faust, *Chief Scientific Officer,* **CEVEC Pharmaceuticals**

Dr Giuliana Vallanti, *Development & Quality Control Director, Qualified Person,* **MolMed**

Workshop Track
(for a maximum of 30 participants)

2.30-4.15pm

DEFINING THE PROS AND CONS OF EU ACCELERATED CLINICAL DEVELOPMENT AND MARKET ACCESS PATHWAYS

- Examining PRiME and the adaptive pathways approach through industry, HTA and regulator lenses

Chair: Dr Gopalan Narayanan, *Vice President, Disruptive Biologics,* **Voisin Consulting Life Sciences (VCLS)**

Rocio Salvador-Roldan, *Policy Officer, DG Health,* **European Commission**

Dr Toby Toward, *Head of Commercial Strategy,* **Immunocore**

Dr Nick Crabb, *Programme Director, Scientific Affairs, Centre for Health Technology Evaluation* **National Institute for Health & Care Excellence**

Anne-Virginie Eggimann, *Vice President, Regulatory Science,* **bluebird bio**

4.15 **Afternoon tea in the exhibition area**

Track 1: Cell-based therapy business & innovation / Track 3: Vector business & innovation

4.45-6.15pm

MEANINGFUL DE-RISKING STRATEGIES AROUND ATMP RAW AND STARTING MATERIAL QUALITY AND SUPPLY

- How to manage risk in your raw material supply chain along the full life cycle of a molecule, from research through to commercial scale?
- How good is good enough? Can we define 'adequate' quality of materials (including donor cells) to meet regulators' requirements in Europe and further afield?

Chair: Wilfried Dalemans, *Chief Technical Officer,* **TiGenix**

Dr Christiane Niederlaender, *CAT Member; Senior Quality Assessor,* **UK Medicines and Healthcare Products Regulatory Agency (MHRA)**
Dr Knut Niss, *Vice President, Program Management,* **Mustang Bio**
Dr Robert Deans, *CTO,* **BlueRock Therapeutics**
Dr Sandra van Wetering, *COO,* **DCPrime BV**

Track 2: R&D updates & emerging science

4.45-6.15pm

EMERGING CELLULAR IMMUNOTHERAPY: ATTACKING SOLID TUMOURS

- Case studies: How is improving understanding of the tumour microenvironment driving novel cellular immunotherapy development?
  - Modulating the tumour microenvironment to inhibit tumour growth - determining immune suppressive pathways to predict response to immunotherapies
  - Targeting Neo-Antigens for more effective immuno-oncology treatment
  - Engaging the immune system in cold tumours: what work is being done to fully understand how to turn a cold tumour hot?

Dr David Gilham, *Vice President R&D,* **Celyad**
Dr Cedrik Britten, *Head, Cellular Therapy- Immuno-Oncology,* **GlaxoSmithKline**
Jan Spanholtz, PhD, *Chief Scientific Officer,* **Glycostem**

6.15 **Close of day 1, followed by a drinks reception**

AGENDA

DAY 2: THURSDAY, SEPTEMBER 21ST 2017

7.30 REGISTRATION & BUFFET BREAKFAST IN THE EXHIBITION AREA

Opening morning plenary

FINDING A NEW WAY: MARKET ACCESS STRATEGIES TO ENSURE EUROPEAN PATIENTS BENEFIT FROM CELL, GENE AND IMMUNOTHERAPY INNOVATION

9.00 CHAIR'S INTRODUCTION

9.05 PUBLIC SECTOR AND INDUSTRY PERSPECTIVES

- Affordability: how are major European national health systems evolving with regard to their ability/willingness to accommodate novel, premium-priced biotherapeutics?
- Identifying key likely reimbursement models for now and the future: which ones can work for manufacturers, healthcare systems and patients alike?
- What might the reimbursement model of the future look like for CAR T cell immunotherapies, including in the combination therapy scenario?
- What will European market access strategies for CAR T cell therapies look like from the industry perspective?
- How should pharma and biotech assemble their value propositions and data to help ensure patient access to curative therapies, in HTAs/payers' eyes?
- What premium-priced biologic pricing and reimbursement lessons can the new wave of biotherapeutics take from the mAbs pharma giants?

Dr Tay Salimullah, *Global Pricing & Market Access Director,* **Novartis**
Dr Nick Crabb, *Programme Director, Scientific Affairs,* **Centre for Health Technology Evaluation National Institute for Health & Care Excellence**
Dr Hans Middelhoven, *Head Innovative Pricing, Global Pricing and Market Access,* **F-Hoffman- La Roche**

10.10 DRAWING LESSONS FROM ATMPS COMMERCIALISED TO DATE TO INFORM FUTURE MARKET ACCESS STRATEGIES
Angela Crossman, *Global Market Access Director, Gene Therapy,* **GlaxoSmithKline**
Dr Diego Ardigo, *Project Leader, ATMP's,* **Chiesi Farmaceutici SpA**
Dr Detlev Parow, *Head of Department, Care Management Development,* **DAK-Gesundheit - Germany**
Dr Luca Alberici, *Director of Business Development,* **MolMed**

10.40 FOCUSING IN ON COST OF GOODS – WHAT IMPROVEMENTS HAVE BEEN - AND CAN BE MADE?
Featuring a summary of the key conclusions and outputs drawn from Phacilitate's Special Interest Group on Automation (staged in May 2017)
Moderator: Dr Cenk Sumen, *Senior Manager, Business Development,* **PCT**
Dr Damian Marshall, *Head of Analytical Development,* **Cell and Gene Therapy Catapult**
Dr Stefano Baila, *Industrialization Manager,* **Celyad**

11.00 CLOSE OF SESSION – MORNING COFFEE IN THE EXHIBITION AREA

Followed by your choice of highly interactive breakout tracks:

Track 1: Cell-based therapy business & innovation

11.30am-1.00pm

HARNESSING THE CUTTING EDGE OF AUTOMATION TO DRIVE ROBUST, COST EFFECTIVE CELL-BASED THERAPY MANUFACTURING AT COMMERCIAL SCALE

- How and where to maximise the impact of automation on ATMP scale up/out strategies in Europe?

Chair: Matthew Durdy, *Chief Business Officer,* **Cell and Gene Therapy Catapult**

Dr Lothar Germeroth, *Senior Vice President,* **Juno Therapeutics, Inc;** *Managing Director,* **Juno Therapeutics GmbH**

Sharon Grimster, *General Manager, Wales,* **ReNeuron**

Dr Hicham Majd, *Device Manager - Device Technology, Biologics Technical Development and Manufacturing (BTDM),* **Novartis Pharma AG**

Dr Maya Fürstenau-Sharp, *Field Marketing Manager EU, Regenerative Medicine,* **Sartorius Stedim Biotech GmbH**

Track 2: R&D updates & emerging science / Track 3: Vector business & innovation

11.30 - 1.00pm

NON-VIRAL DELIVERY PLATFORMS – DOES THE LATEST DATA SUPPORT WIDESPREAD EX VIVO AND IN VIVO GENE THERAPY APPLICATION?

- Assessing the latest non-viral delivery technologies - can we define their real potential to deliver improvements on viral alternatives and/or to open up new indications and markets to gene therapy?

Alexander Scheer, PhD, *Chief Scientific Officer,* **Erytech**

Sharron Gargosky, PhD, *Chief Clinical & Regulatory Officer,* **OncoSec Medical Incorporated**

Dr Niranjan Sardesai, *Chief Operating Officer,* **Inovio Pharmaceuticals, Inc**

Dr Madhusudan Peshwa, *Chief Scientific Officer, Executive Vice President, Cellular Therapies,* **MaxCyte, Inc**

Workshop Track *(for a maximum of 30 participants)*

11.30 - 1.00pm

BIOTECH EXIT STRATEGY BOOT CAMP PART 1: PHARMA DEAL-MAKING

A case study-driven workshop on how to initiate and structure pharma-biotech deals.

- When is the timing right for you?
- What are the pros, cons and pitfalls in partnering at the various stages of R&D?
- What are the key items on pharma 'must-have' and 'must-avoid' checklists when considering a biotech partner?
- Intellectual Property through pharma eyes: how much value does pharma see in IP (vs. the potential of being first to market, for example)? What can/should be patented? How strong is your IP in pharma's eyes, or how open to challenge?

Dr Sven Kili, *Vice President & Head of Cell & Gene Therapy Development, Rare Disease Unit,* **GlaxoSmithKline**
Ajan Reginald, *CEO,* **Celixir**
Dr Eric Soller, *Vice President of Corporate Development & Strategy,* **BlueRock Therapeutics**

1.00 **Buffet lunch in the exhibition area**

AGENDA

Track 1: Cell-based therapy business & innovation

2.00pm-3.30pm

WHAT TOOLS DO CELL THERAPY MANUFACTURERS NEED TO DELIVER NECESSARY TIME AND COST SAVINGS IN QC/RELEASE TESTING?

- Meeting European regulators' expectations relating to cell therapy characterisation and quality
- What will tomorrow's bioanalytical toolbox for cell-based therapy look like?

Chair: Dr Christopher Bravery, *Director,* **Advanced Biologicals Ltd**

Dr Christiane Niederlaender, *CAT Member; Senior Quality Assessor,* **UK Medicines and Healthcare Products Regulatory Agency (MHRA)**

Dr Robert Deans, *CTO,* **BlueRock Therapeutics**

Dr Stefano Baila, *Industrialization Manager,* **Celyad**

Pierre Heimendinger, PharmD, *Head of Industrial Development,* **TxCell**

Track 2: R&D updates & emerging science

2.00pm-3.30pm

GENE EDITING PLATFORMS

- Regulatory and safety challenges posed by new gene and cell therapy technologies
- Case studies: what is the latest data from the non-clinical and clinical application of gene editing platforms in both ex vivo and in vivo gene therapy settings?

Dr Madhusudan Peshwa, *Chief Scientific Officer, Executive Vice President,* **Cellular Therapies, MaxCyte, Inc**

Dr Tim Farries, *Director, Regulatory Affairs, Cell & Gene Therapies,* **ERA Consulting**

Seokjoong Kim, PhD, *Research Director,* **ToolGen, Inc**

Track 3: Vector business & innovation

2.00pm-3.30pm

VIRAL VECTOR BIOANALYTICS AND QUALITY CONTROL: ALLEVIATING UNCERTAINTY THROUGH REGULATORY INSIGHT AND TECHNICAL INNOVATION

- Sharing practical manufacturer and regulatory experiences to define appropriate 'minimum' requirements in viral vector characterisation and QC terms at early and late clinical phases

THEN

HOW ARE NEXT-GENERATION VIRAL VECTOR ENGINEERING APPROACHES ADDRESSING LONG-STANDING TARGETED DELIVERY AND SAFETY CONCERNS?

- Case studies exploring:
  - Novel designs and approaches for re-targeting vectors
  - Novel vector surface engineering

Chair: Dr Jan Thirkettle, *CEO & Chief Development Officer,* **Freeline Therapeutics**
Dr David Venables, *CEO,* **Synpromics**
Dr Fraser Wright, *Co-Founder & Chief Technology Officer,* **Spark Therapeutics** *(provisionally confirmed)*
Dr Janneke Meulenberg, *COO,* **Arthrogen**

Workshop Track *(for a maximum of 30 participants)*

2.00pm-3.30pm

BIOTECH EXIT STRATEGY BOOT CAMP PART 2: IPO

- A journey through the fundamentals of IPO, both within Europe and worldwide.
- How to assess if/when/where IPO is right for you?
- What are the 'need to knows' in terms of how IPOs work, and what the full range of consequences are for the biotech organisation? (How to ensure you receive analyst coverage on an ongoing basis as a European biotech company on NASDAQ, for instance?)
- Benefit from the insights of both financial market experts and industry leaders who have been there and done it

Eric Dutang, *CFO,* **Cellectis**
Patrick Jeanmart, *CFO,* **Celyad**
Dr Helen Tayton-Martin, *Chief Business Officer* **Adaptimmune**

3.30 **Afternoon tea in the exhibition area**

Track 1: Cell-based therapy business & innovation / Track 3: Vector business & innovation

4.00pm-5.30pm

A SHIFT IN MINDSET? INTEGRATED, END-TO-END SUPPLY CHAIN AND SUPPORT SYSTEM PLANNING TO UNLOCK EUROPEAN ATMP MARKET POTENTIAL

- How to alleviate the impact of costly, time consuming cell therapy supply chains on industry, payer and patient alike?

John Lunger, *Vice President, Manufacturing & Supply Chain,* **Adaptimmune**
Sabine Schöffel-Weiß, *Deputy Director,* **CMS Cellex Medical Services GmbH**
Dr Matthew Lakelin, *Vice President,* **Scientific Affairs, TrakCel**
Wilfried Dalemans, *Chief Technical Officer,* **TiGenix**

Track 2: R&D updates & emerging science

4.00pm-5.30pm

ONCOLYTIC VIRUSES AND CANCER VACCINES: WHAT IS ON THE HORIZON?

- Case studies: Clinical progress in oncolytic virus development - is this modality going to usher a new age of cancer treatment? What does the emerging data show?
- Case studies: Reviewing the past to protect the future - what progress has been made to ensure next generation cancer vaccines show clinical success

Dr Andres Gutierrez, *Chief Medical Officer,* **Oncolytics Biotech Inc**
Dr Niranjan Sardesai, *Chief Operating Officer,* **Inovio Pharmaceuticals, Inc**
Dr Richard Janeczko, *Chief Executive Officer,* **VEL Vaccines Biosciences Inc**

5.30 **Close of Phacilitate Cell & Gene Therapy Europe 2017**

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