ExpandedAccessSummit

EA3

AGENDA

EXPANDED ACCESS 3.0

The Third Annual Expanded Access Summit®
January 27-29, 2020
National Press Club | Washington DC

SUMMARY:

MONDAY, JANUARY 27 (Evening)

6:00 – 9:00pm | The Pre-Workshop: Module A
Global Regulation and Industry Best Practices

6:00 – 6:15pm
Evening Check-in and Networking

6:15 – 6:30pm
WHY WE HAVE PRE-MARKET ACCESS TO MEDICINES: *An introduction*
- **Marjorie Speers, PhD,** Executive Director, Clinical Research Pathways

6:30 – 7:00pm
U.S. EXPANDED ACCESS REGULATION: *30+ years of regulation plus recent updates including Right To Try and Project Facilitate*
- **Richard Klein,** Former Director, Patient Liaison Program, FDA
- **Amy Barone, MD,** Medical Officer, Oncology Center of Excellence, FDA

7:00 – 7:45pm
GLOBAL REGULATORY LANDSCAPES: *Europe, Americas, Asia*
- **David Schwicker,** Principal and Founder, ORPHA Strategy Consulting
- **Connie Coulomb, MBA,** Managing Partner, Coulomb Strategy Consulting
- **Georges van Baelen, MScPh,** Founder and President, Arctiryon, Inc.

7:45 – 7:55pm
REGULATORY Q&A REVIEW
- **All Speakers**

7:55 – 8:10pm
Break

8:10 – 8:35pm
THE RANGE OF USES FOR EXPANDED ACCESS: *Pharmaceuticals, devices, research-stage, new market, new formulation, etc.*
- **Karen Frascello,** Director, Global Medical Affairs, Alnylam Pharmaceuticals
- **Kevin Weatherwax,** Managing Director, University of Michigan Health System

8:35 – 9:00pm
BUSINESS AGREEMENTS AND IRB COORDINATION WITH HEALTH CARE PROVIDERS
- **Erika Segear, PhD, RAC,** Associate Director of Regulatory Affairs, Duke University School of Medicine
- **Chris Beardmore,** CEO, Anova LLC

9:00pm
Evening Workshop Adjourned

EXPANDED ACCESS 3.0

January 27-29, 2020

EA3

TUESDAY, JANUARY 28 (Morning)

8:15am – 5:00pm | Module 1: The Global Stakeholder Forum
Integrating Expanded Access into the Drug Development Process

7:30 – 8:15am
CHECK IN, NETWORKING, BREAKFAST

8:20 – 8:30am
OPENING REMARKS

- **Jess Rabourn, CFA,** CEO, WideTrial Inc.

8:30 – 9:30am: Session 1
MEETING THE ORIGINAL PROMISE OF EXPANDED ACCESS: *How far have we come? Are we on the right track?*

- **Steve Usdin,** Senior Editor, BioCentury*
- **Tony Fauci, MD,** Director, National Institute of Allergy and Infectious Diseases (NIAID), NIH
- **Peter Barton Hutt,** Senior Counsel, Covington & Burling LLP
- **Marjorie Speers, PhD,** Executive Director, Clinical Research Pathways

9:30 – 10:05am: Session 2
MORNING KEYNOTE ADDRESS: *Smarter, More Inclusive Drug Development*

- **Janet Woodcock, MD,** Director, Center for Drug Evaluation and Research (CDER), FDA

10:05 – 10:30am
Break

10:30 – 11:30am: Session 3
GETTING PAST THE SIMPLE NARRATIVES: *Deeper looks at the opportunities, obstacles, politics, and competitive interests surrounding pre-market access*

- **Marc Boutin,** Chief Executive Officer, National Health Council*
- **Anne Cropp, PharmD,** Chief Scientific Officer, Early Access Care, LLC
- **Jonathan Darrow, SJD,** Faculty Member, Harvard Medical School
- **Richard Klein,** Former Director, Officer of Health and Constituent Affairs, FDA (1990-1997)
- **Tobias Polak, PhD Candidate,** Erasmus University

11:30am – 12:30pm: Session 4
FRAMEWORKS FOR REIMBURSING EXPLORATORY TREATMENT FOR SERIOUS & LIFE-THREATENING HEALTH CONDITIONS:
Re-examining "Reasonable and Necessary" care options; coverage with evidence generation

- **Jess Rabourn, CFA,** CEO, WideTrial Inc.*
- **Alexander Natz,** Secretary General, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)
- **Ed Pezalla, PhD, MPH,** Former National Medical Director and VP, Aetna (2007 – 2016)
- **Peter Pitts,** President, Center for Medicine in the Public Interest

EXPANDED ACCESS 3.0

January 27-29, 2020

EA3

TUESDAY, JANUARY 28 (Afternoon)

8:15am – 5:00pm | Module 1: The Global Stakeholder Forum
Integrating Expanded Access into the Drug Development Process

12:30 – 1:15pm
Buffet Lunch

1:15 – 1:45pm: Session 5
AFTERNOON KEYNOTE: *Clinical discovery through treatment of wider ranges of patients*

- **Chris Austin, MD,** Director, National Center for Advancing Translational Sciences, NIH

1:45 – 3:00pm: Session 6
THE INTEGRATION OF TREATMENT AND CLINICAL RESEARCH: *Informing and strengthening the clinical development of novel regulated products through data-generating treatment programs*

- **Paul Aliu, PharmD,** Global Head Medical Governance, Novartis Pharmaceuticals
- **Amy Barone, MD,** Medical Officer, Oncology Center of Excellence, FDA
- **Stuart Bell, PhD,** Vice President, Consulting, Inceptua Medicines Access
- **Suanna Bruinooge, MPH,** Director of Research Strategy, CENTRA, ASCO
- **Michael Kurilla, MD, PhD,** Director, Clinical and Translational Science Awards Program (CTSA), National Center for Advancing Translational Sciences (NCATS), NIH

3:00 – 3:10pm
Break

3:10 – 4:10pm: Session 7
HEALTH CARE PROVIDERS AND LIFE SCIENCE COMPANIES PARTNERING FOR WIDER ACCESS: *Identifying the beneficiaries of pre-market access, and designing appropriate, sustainable partnerships*

- **Chris Beardmore,** CEO, Anova LLC*
- **Erika Segear, PhD, RAC,** Associate Director of Regulatory Affairs, Duke University School of Medicine
- **Georges van Baelen, MScPh,** Founder and President, Arctiryon, Inc.
- **Kevin Weatherwax,** Managing Director, Michigan Institute for Clinical and Health Research, University of Michigan Health System

4:10 – 5:00pm: Session 8
ADDRESSING POPULATION NEEDS: *Pre-market access -including Emergency Use Authorization- to address supply shortages, underserved markets, contagion preparedness, and national defense*

- **Amar Bhat, PhD,** Interim Executive Director, Reagan-Udall Foundation, FDA*
- **John Lagus,** Managing Director of Business Development, Tanner Pharma Group
- **Hilary Marston, MD,** Medical Officer, National Institute of Allergy and Infectious Diseases (NIAID), NIH
- **Robert Walker, MD,** Director, Division of Clinical Development, Biomedical Advanced Research and Development Authority (BARDA), HHS

5:00pm
Session Adjourned

EXPANDED ACCESS 3.0

January 27-29, 2020

EA3

TUESDAY, JANUARY 28 (Evening)

5:15pm – 8:00pm | Grand Reception

- **Gourmet Food and Beverage**
- **Exhibitor Galleries and Poster Session**
- **Music, Ping Pong Tournament, Additional Activities**

6:00pm – 8:00pm | Module B: The Innovators Showcase
Real Solutions Presented by Leaders in the Field

6:00 – 6:30pm
Set 1

- **MyTomorrows |** Dennis Akkaya, Director of Corporate Development
- **Caligor Coghlan |** Geoff Fatzinger, Director of Market Access
- **Early Access Care |** Anne Cropp, PharmD, Chief Scientific Officer
- **Tanner Pharma Group |** Paul Stanton, Global Commercial Director

6:30 – 6:45pm
Break

6:45 – 7:15pm
Set 2

- **xCures |** Mike Newton, CEO
- **WideTrial |** Jess Rabourn, CEO
- **Anova |** Martin Walsh, President and Co-founder
- **CISCRP |** Behtash Bahador, Associate Director

7:15 – 7:30pm
Break

7:30 –8:00pm
Set 3

- **The Global Guide to Compassionate Use Programs|** Sean Turbeville, PhD, Director
- **The Accreditation Council for Medical Affairs |** William Soliman, PhD, BCMAS, Chairman
- **EA, RTT, and Payer Policy |** Jacob Hill, Franklin University, Supply Chain Management Program

8:00pm
Showcase Adjourned

EXPANDED ACCESS 3.0

EA3

January 27-29, 2020

WEDNESDAY, JANUARY 29

8:30am – 3:00pm | Module 2: The Interactive Forum
Charting the Course for Modern Clinical Development Speakers

8:00 – 8:30am
NETWORKING AND BREAKFAST

8:30 – 8:40am
RECAP OF DAY 1

- **Jess Rabourn, CFA,** CEO, WideTrial Inc.

8:40 – 9:45am: Session 1
INCLUSIVITY IN CLINICAL DEVELOPMENT: *Should all medically suited patients be provided the option to participate in the development of a new medicine? If so, how should this be accomplished? What are the barriers?*

- **Behtash Bahador, MS,** Associate Director, Relationship Management and Development, Center for Information & Study on Clinical Research Participation (CISCRP)*
- **Naomi Lopez Bauman,** Director of Healthcare Policy, Goldwater Institute
- **Christina Hartman, MPH,** Senior Director of Advocacy, The Assistance Fund
- **Andrew Shuman, MD, FACS,** Chief of Clinical Ethics Services, University of Michigan Medical School

9:45 – 10:00am
Break

10:00 – 11:00am: Session 2
EXPANDED ACCESS FOR MODERN HIGH-COST THERAPEUTICS: *New challenges with cutting edge biologics, including cell & gene therapies, nucleotide, and novel antibodies*

- **Khrystal K. Davis,** Founder & President, Texas Rare Alliance & Texas Newborn Screening Advisory Committee Member*
- **Karen Frascello,** Director, Global Medical Affairs, Early Access Programs, Alnylam Pharmaceuticals
- **Jeff Leider,** President, X2, "Let Them Be Little" Foundation
- **Douglas Sproule, MD,** Vice President, Spinal Muscular Atrophy Therapeutic Area Head, AveXis

11:00am – 12:00pm: Session 3
WHO SHOULD PAY FOR PRE-MARKET TREATMENT ACCESS PROGRAMS: *If Cost Recovery is key to feasible access programs, then who should the costs be recovered from and under what circumstances?*

- **Marjorie Speers, PhD,** Executive Director, Clinical Research Pathways*
- **Marc Boutin,** Chief Executive Officer, National Health Council
- **David Farber, JD,** Senior Partner, King & Spalding

12:00 – 12:15pm
INTRODUCTION TO THE INTERACTIVE CHALLENGE

- **Behtash Bahador, MS,** Associate Director, Relationship Management and Development, Center for Information & Study on Clinical Research Participation (CISCRP)*

12:15 – 1:15pm
Box Lunch

1:15 – 2:30pm: Session 4
THE INTERACTIVE CHALLENGE; BREAKOUT AND REPORT
Assigned seating at tables representing model stakeholder organizations. Delegates present findings.

- **Behtash Bahador, MS,** Associate Director, Relationship Management and Development, Center for Information & Study on Clinical Research Participation (CISCRP)*
- **William Hoos, MS, MBA,** Chief Commercial Officer, xCures*

2:30 – 3:00pm: Session 5
POLICY RECOMMENDATIONS / RESOLUTIONS: *A moderated roundtable discussion with audience*

- **Paul Aliu, PharmD,** Global Head Medical Governance, Novartis Pharmaceuticals
- **Pat Furlong,** Founding President and CEO, Parent Project Muscular Dystrophy (PPMD)
- **Peter Pitts,** President, Center for Medicine in the Public Interest
- **Steve Usdin,** Senior Editor, BioCentury

** denotes the Discussion Leader of each panel session*

EXPANDED ACCESS 3.0

January 27-29, 2020

EA3

SPEAKER FACULTY

Paul Aliu, PharmD, Global Head Medical Governance, Novartis Pharmaceuticals

Chris Austin, MD, Director, National Center for Advancing Translational Sciences, NIH

Georges van Baelen, MScPh, Founder and President, Arctiryon, Inc.

Behtash Bahador, MS, Associate Director, Relationship Management and Development, Center for Information & Study on Clinical Research Participation (CISCRP)

Amy Barone, MD, Medical Officer, Oncology Center of Excellence, FDA

Naomi Lopez Bauman, Director of Healthcare Policy, Goldwater Institute

Chris Beardmore, CEO, Anova LLC

Stuart Bell, PhD, Vice President, Consulting, Inceptua Medicines Access

Amar Bhat, PhD, Interim Executive Director, Reagan-Udall Foundation, FDA

Marc Boutin, Chief Executive Officer, National Health Council

Suanna Bruinooge, MPH, Director of Research Strategy, CENTRA, ASCO

Anne Cropp, PharmD, Chief Scientific Officer, Early Access Care, LLC

Connie Coulomb, MBA, Managing Partner, Couloumb Strategy Consulting

Jonathan Darrow, SJD, Faculty Member, Harvard Medical School

Khrystal K. Davis, Founder & President, Texas Rare Alliance & Texas Newborn Screening Advisory Committee Member

David Farber, JD, Senior Partner, King & Spalding

Tony Fauci, MD, Director, National Institute of Allergy and Infectious Diseases (NIAID), NIH

Karen Frascello, Director, Global Medical Affairs, Early Access Programs, Alnylam Pharmaceuticals

Pat Furlong, Founding President and CEO, Parent Project Muscular Dystrophy (PPMD)

Christina Hartman, MPH, Senior Director of Advocacy, The Assistance Fund

William Hoos, MS, MBA, Chief Commercial Officer, xCures

Peter Barton Hutt, Senior Counsel, Covington & Burling LLP

Richard Klein, Former Director, Officer of Health and Constituent Affairs, FDA (1990-1997)

Michael Kurilla, MD, PhD, Director, Clinical and Translational Science Awards Program (CTSA), National Center for Advancing Translational Sciences (NCATS), NIH

John Lagus, Managing Director of Business Development, Tanner Pharma Group

Jeff Leider, President, X2, "Let Them Be Little" Foundation

Hilary Marston, MD, Medical Officer, National Institute of Allergy and Infectious Diseases (NIAID), NIH

Alexander Natz, Secretary General, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)

Ed Pezalla, PhD, MPH, Former National Medical Director and VP, Aetna (2007 – 2016)

Peter Pitts, President, Center for Medicine in the Public Interest

Tobias Polak, PhD Candidate, Erasmus University

Jess Rabourn, CFA, CEO, WideTrial Inc.

David Schwicker, Principal and Founder, ORPHA Strategy Consulting

Erika Segear, PhD, RAC, Associate Director of Regulatory Affairs, Duke University School of Medicine

Andrew Shuman, MD, FACS, Chief of Clinical Ethics Services, University of Michigan Medical School

Marjorie Speers, PhD, Executive Director, Clinical Research Pathways

Douglas Sproule, MD, Vice President, Spinal Muscular Atrophy Therapeutic Area Head, AveXis

Steve Usdin, Senior Editor, BioCentury

Robert Walker, MD, Director, Division of Clinical Development, Biomedical Advanced Research and Development Authority (BARDA), HHS

Kevin Weatherwax, Managing Director, Michigan Institute for Clinical and Health Research, University of Michigan Health System

Janet Woodcock, MD, Director, Center for Drug Evaluation and Research (CDER), FDA

(In Memory) **Frank Young, MD, PhD,** Former FDA Commissioner (1984-1989)