INC
CRITICAL PATH INSTITUTE

International Neonatal Consortium

SEPTEMBER 12-13, 2016

APPLYING REGULATORY SCIENCE TO NEONATES

Second Annual Scientific Workshop at EMA

European Medicines Agency | Canary Wharf, London

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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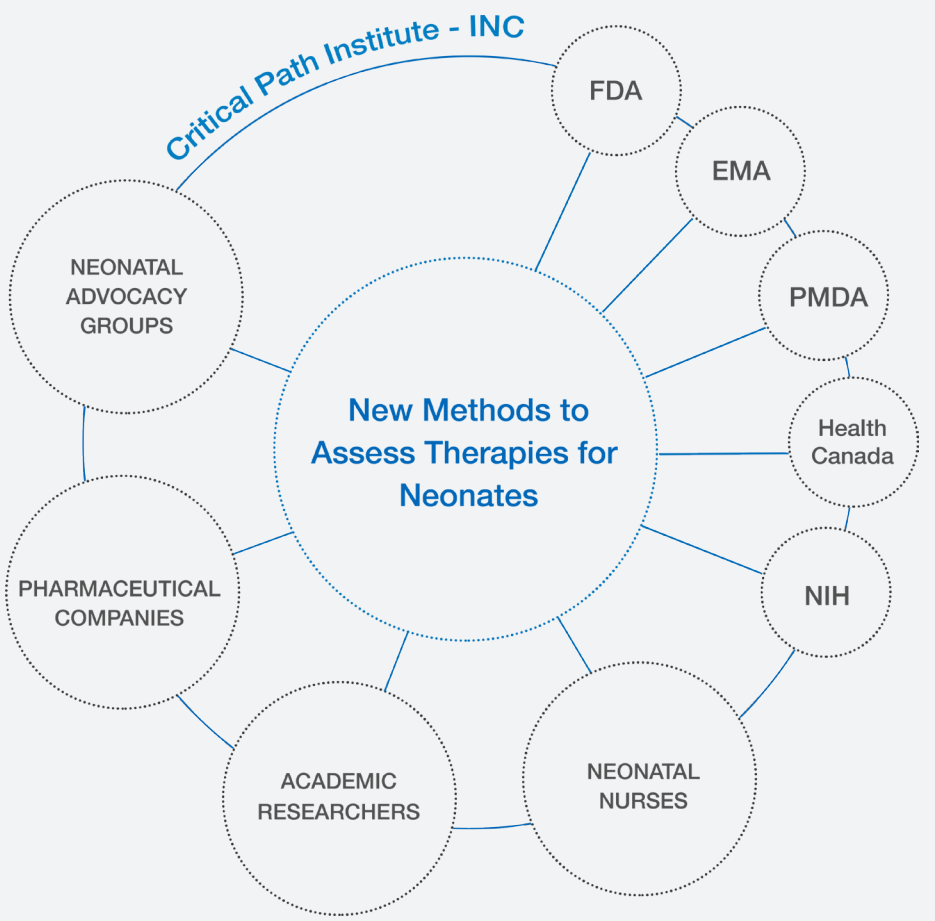
Introduction

Welcome to the Second Workshop on *Applying Regulatory Science to Neonates* at the European Medicines Agency. The first workshop at EMA brought together a global array of stakeholders to launch the International Neonatal Consortium (May 18-19, 2015). Apart from reporting on the consortium's collaborative efforts, INC workshops serve to identify and prioritize projects designed to accelerate the development of therapies for neonates.



http://c-path.org/programs/inc/

MEMBERS

Neonatal Nurses

- COINN
- NANN

Companies

- Chiesi Pharmaceuticals
- Eli Lilly
- Johnson & Johnson
- Novartis Pharmaceuticals
- Parabase Genomics
- Pfizer
- Sanofi Pharmaceuticals
- Shire
- Therabron Therapeutics

Families/Advocacy

- BLISS
- EFCNI-Consultant
- Graham's Foundation
- NEC Society
- Preemie Parent Alliance

INC focuses on therapeutic areas most commonly encountered in neonatal care: neonatal lung, brain, and gastrointestinal injury, retinopathy of prematurity, sepsis, hemodynamic adaptation, and neonatal abstinence syndrome. Affecting both term and preterm neonates, these conditions are associated with significant short and long term morbidity and mortality. Preventing preterm labor is another consortium interest.

INC AND THE NICU

The International Neonatal Consortium will concentrate its efforts on those conditions most commonly encountered in NICUs, and on the prevention of preterm birth.

RETINOPATHY OF PREMATURITY (ROP)

Many preterm infants develop ROP, with more severe forms requiring treatment with laser ablation or anti-angiogenic agents (off-label use). The risk of visual impairment or blindness in this high-risk population remains unacceptably high. New approaches to the prevention and treatment are urgently needed.

HEMODYNAMIC ADAPTATION

An international consensus on acceptable blood pressure for preterm and term newborns is considered a cross-cutting need that could form the basis of inclusion criteria for drug study protocols in the neonatal period. Both low and high blood pressure thresholds need to be defined along with standard methods of measurements in different health care settings.

NEONATAL LUNG INJURY AND CIRCULATORY FAILURE

Bronchopulmonary Dysplasia (BPD) is the most common sequela of neonatal intensive care, most often occurring in preterm infants. Infants with BPD can develop repeated pulmonary infections, pulmonary hypertension, and asthma. Novel approaches to the prevention and treatment of BPD and the resulting chronic respiratory morbidity are urgently needed. Term infants can develop Persistent Pulmonary Hypertension of the Newborn (PPHN) when the normal transition of the fetal circulation does not occur at birth and during severe respiratory failure. These infants continue to be extremely ill despite the use of high-frequency ventilation, exogenous surfactant, and inhaled nitric oxide (iNO), and would clearly benefit from new treatment modalities. Defining optimal treatment options will require better definitions and validation of endpoints for assessment of pharmacologic interventions.

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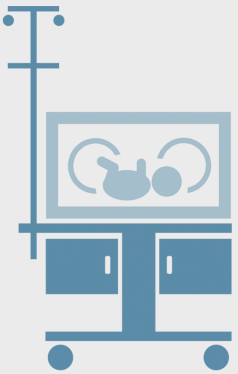

PERINATAL/NEONATAL INFECTIONS

A significant number of preterm and term infants will develop serious bacterial and viral infections resulting in death or NDI. Over 25% of preterm infants will develop early-onset or nosocomial sepsis, with an increasing number of infections resistant to traditional antibiotic and antiviral agents.

NEONATAL ABSTINENCE SYNDROME (NAS)

There has been a dramatic increase in the incidence of NAS resulting from *in utero* exposure to opiates, with rates tripling in the last 10 years. There is significant uncertainty on whom to treat, when to treat, and how to treat affected infants. In-depth studies with better assessment techniques and short- and long-term outcome measures are urgently needed.

DRUGS TO PREVENT PRETERM LABOR

The US continues to have the highest rates of prematurity of any developed country, with annual costs exceeding $29 billion. Considerable research into possible genetic and/or environmental factors has failed to establish a definitive pathogenesis and few treatments have made a significant impact (the incidence remains at >10%). Prevention strategies involving cerclage and progesterone have not been as successful as previously hoped. New therapeutic approaches to significantly reduce preterm birth would be expected to result in dramatic improvements in infant mortality and outcome.

NEONATAL GASTROINTESTINAL INJURY

Between 5 and 10% of all extremely preterm infants will develop Necrotizing Enterocolitis (NEC), a devastating illness associated with death, short gut syndrome, and NDI. Despite significant research on breast milk, probiotics, and other novel agents (e.g., lactoferrin, prebiotics) the incidence of NEC has not substantially changed in the last 20 years and represents a leading cause of late mortality for preterm infants.

NEONATAL BRAIN INJURY

Term infants continue to develop encephalopathy, stroke, and seizures around the time of birth. Despite the use of newer anti-seizure drugs and brain cooling, morbidity and mortality remain unacceptably high. In preterm infants, the prevention and treatment of severe intraventricular haemorrhage (IVH) and white matter injury (WMI) is crucial. These conditions are leading factors in the development of significant neurodevelopmental impairment (NDI).

Meeting Objectives

- Explore approaches that the neonatal community can take to embrace a research culture.
- Review the progress of the INC Workgroups and introduce two new workstreams.
- Discuss what clinical pharmacology can contribute to the appropriate use of narcotics for sedation, analgesia, and/or neonatal abstinence syndrome (NAS).
- Examine how studies on precision medicine for neonates—both pharmacogenetics and long-term clinical outcomes—should be designed to maximize benefits.
- Identify regulatory science projects that INC could take on to improve the treatment of necrotizing enterocolitis (NEC).

Agenda

Monday, September 12

9:00 am ***Welcome***
GUIDO RASI (European Medicines Agency)

9:15 am ***Keynote:*** *Where We Need to Move Neonatology*
NEENA MODI (Imperial College London)

9:45 am ***Session I:*** *Embracing a Research Culture*
GERRI BAER (US Food and Drug Administration), Co-Chair
MARY SHORT (Eli Lilly), Co-Chair

Fostering a Culture of Research to Improve Outcomes
KELLY WADE (Children's Hospital of Philadelphia)

10:15 am – 10:45 am COFFEE BREAK

10:45 am ***Session I Panel:*** *Fostering a Culture of Research: Beliefs, Strengths/Barriers, Needs, INC Role/Opportunity for Establishing a Research Culture*
MEHALI PATEL (Bliss)
JENNIFER CANVASSER (Preemie Parent Alliance)
YANNIC VERHAEST (European Foundation for the Care of Newborn Infants)
CATHERINE SHERWIN (University of Utah)
CAROLE KENNER (Council of International Neonatal Nurses)
WAKAKO EKLUND (National Association of Neonatal Nurses)
JORDI LLINARES-GARCIA (European Medicines Agency)
NATHALIE SEIGNEURET (The Innovative Medicines Initiative/University Hospital Zurich)
CHRISTINA BUCCI-RECHTWEG (Novartis)

Projects to consider for a Research Culture Workstream

1. Develop best practices document (evidence-based when possible) to guide informed-consent process for neonatal research.

2. Analysis/mapping of the resources that currently exist (along with needs) to educate and support families in decision-making with regards to research.

3. Enhancing communication and public relations around neonatal research.

4. Family-centered research embedded in family-centered NICU care– facilitating parents' ability to choose research that is best for their child/family.

5. Increased involvement of former NICU parents and graduates, nurses, and other multidisciplinary team members in the design and planning of research.

6. Enhance nursing education/orientation in regards to research.

12:00 pm – 1:00 pm LUNCH

1:00 pm – 2:15 pm ***Session II:*** *INC Workgroup Updates and New INC Workstreams*

RON PORTMAN, INC Co-Director (Novartis)
Clinical Pharmacology: BOB WARD (University of Utah)
Seizures: JANET SOUL (Harvard University)
Bronchopulmonary Dysplasia: ROBIN STEINHORN (Children's National Hospital)
Data: TOM DIACOVO (Columbia University)

2:15 pm – 3:00 pm ***Session III:*** *Use of Narcotics for Sedation, Analgesia, or Treatment of Neonatal Abstinence Syndrome*

JOHN VAN DEN ANKER (Children's National Health System/University Children's Hospital Basel), Co-Chair
JON DAVIS, INC Co-Director (Tufts University), Co-Chair

The Opioid Epidemic and Neonatal Abstinence Syndrome

STEPHEN PATRICK (Vanderbilt University School of Medicine)

The Use of Narcotics for Sedation or Analgesia

JACOB ARANDA (University Hospital of Brooklyn)

3:00 pm – 3:30 pm COFFEE BREAK

3:30 pm – 5:00 pm ***Session III Panel:***

JACOB ARANDA (University Hospital of Brooklyn)
GERRI BAER (US Food and Drug Administration)
ANNE GREENOUGH (King's College London)
WALTER KRAFT (Thomas Jefferson University)
STEPHEN PATRICK (Vanderbilt University School of Medicine)
MERRAN THOMSON (Hillingdon Hospital NHS Trust, Chiesi)
ROBERT WARD (University of Utah)
MAREK MIGDAL (PDCO - European Medicines Agency)

Projects to consider for a Neonatal Abstinence Syndrome Workstream

1. To develop a gold standard definition of neonatal abstinence syndrome and/or neonatal opioid withdrawal syndrome based upon objective validated tools.

2. To develop standardized outcome and process measures for NAS/NOWS to be utilized in clinical trials.

3. To understand the impact of non-pharmacologic treatment in optimizing outcomes for women and infants impacted by opioid use disorder and NAS, including rooming in, soothing techniques, and breastfeeding, in addition to, and independent of, various pharmacotherapies for NAS.

4. To develop a gold standard treatment protocol and standardized medication of choice for severe NAS, address the variability of dosing/drugs, and include defining the safety and role of adjunctive treatments, particularly phenobarbital.

5. To understand the predictors of NAS, both genomic and pharmacologic.

5:00 pm *Concluding Remarks for Day 1*

JON DAVIS, INC Co-Director (Tufts University)

6:30 pm NETWORKING DINNER AT THE PEARSON ROOM

16-19 Canada Square, Canary Wharf

Tuesday, September 13

8:00 am ***Welcome to Day 2***

RALPH BAX (European Medicines Agency)

8:15 am ***Session IV:*** *Precision Medicine for Neonates: Horizon Scanning*

MARK TURNER (University of Liverpool), Chair
ANDY BHATTACHARJEE (Parabase Genomics)
WOLFGANG GÖPEL (University of Lübeck/VOC)
YONGCHANG QIU (Shire)
THOMAS MORGAN (Novartis)
MARISA PAPALUCA (European Medicines Agency)
CYNTHIA POWELL (University of North Carolina) *by WebEx*
STEPHEN SPIELBERG (Drug Information Association)

Projects to consider for a Precision Medicine Workstream

1. Should genetic/pharmacogenetic studies in neonates be the same as or different to other populations?

2. Should genetic/proteomic studies in neonates be the same as or different to other populations?

3. Should the regulatory implications of neonatal genomic/proteomic studies be addressed now or when the field is more mature?

4. What criteria should contribute to quality control of information flow from tests to releasing information to families: which criteria are specific to neonates?

10:00 am – 10:30 am COFFEE BREAK

10:30 am ***Session V:*** *Long-Term Outcomes*

LEX DOYLE (University of Melbourne), Co-Chair
NEIL MARLOW (University College London), Co-Chair

Long-Term Outcomes from Clinical Trials – Why, What, When, and How?
LEX DOYLE (University of Melbourne)

Should Long-Term Outcomes Be the Standard for Neonatal Trials?
NEIL MARLOW (University College London)

Session V Panel:

MARILEE ALLEN (Johns Hopkins University)
PETER ANDERSON (Murdoch Childrens Research Insitite University of Melbourne)
DINA APELE-FREIMANE (PDCO - European Medicines Agency/P. Stradins Clinical University Hospital (Latvia)/MCH Clinic)
SUSAN HINTZ (Stanford University)
SAMANTHA JOHNSON (University of Leicester)
GIANCARLO NATALUCCI (University of Zurich)
A.G. VAN WASSENAER-LEEDMHUIS (Emma Children's Hospital/ Academic Medical Center [AMC], Netherlands)
SUSAN MCCUNE (Office of Translational Sciences, Center for Drug Evaluation and Research - U.S. Food and Drug Administration) *by WebEx*

Projects to consider for a Long-Term Outcomes (LTOs) Workstream

1. Optimal age of recording childhood neurodevelopmental outcomes to support regulatory approval.

2. Optimal definition of childhood neurodevelopmental outcome at that age.

3. Duration of safety monitoring post-regulatory approval.

4. Optimal method of collecting data on LTOs.

5. Potential for using neonatal data as biomarkers for LTO.

12:00 pm – 1:00 pm LUNCH

1:00 pm – 3:00 pm ***Session VI:** Necrotizing Enterocolitis*

RON PORTMAN, INC Co-Director (Novartis), Co-Chair
MICHAEL CAPLAN (University of Chicago), Co-Chair

NEC: State of the Art

MICHAEL CAPLAN (University of Chicago)

Biomarkers and Barriers: Opportunities and Challenges in NEC

KARL SYLVESTER (Stanford University)

Session VI Panel:

TAHA KEILANI (Sigma Tau Pharmaceuticals Inc.)
IRJA LUTSAR (PDCO - European Medicines Agency)
PAOLO MANZONI (S. Anna Hospital, Torino)
TOKUO MIYAZAWA (Showa University, Japan)
JOSEF NEU (University of Florida - Gainesville)
JENNIFER CANVASSER (Necrotizing Enterocolitis (NEC) Society & Preemie Parent Alliance)

Projects to consider for a Necrotizing Enterocolitis in Neonates Workstream

1. Identification and utilization of biomarkers for the early diagnosis of NEC; are there candidates available and what additional investigation is needed?

2. Identification and utilization of biomarkers for the response to treatment of NEC, or possible prognostic indicators.

3. Detailed review and meta-analysis of current methods to prevent and treat NEC in high-risk neonates leading to prioritization and study of leading candidates.

4. Epidemiologic study of NEC across the globe.

5. Determination and clarification of NEC diagnosis: are there different categories that should be considered?

3:00 pm *Concluding Remarks*

MARK TURNER, INC Co-Director

3:15 pm WORKSHOP ADJOURNED

4:00 pm –8:00 pm SATELLITE WORKGROUP SESSIONS TO BE HELD AT THE MARRIOTT WITH A WORKING DINNER

Workgroup Session I on Bronchopulmonary Dysplasia
Workgroup Session II on Data
Workgroup Session III on Seizures

INC Coordinating Committee and Workgroup Members

Coordinating Committee Participants

First Name	Surname	Organization	
Karel	Allegaert	University of Leuven	
Gerri	Baer	US Food and Drug Administration	
Jeffrey	Barrett	Sanofi Pharmaceuticals	
Norman	Barton	Shire	
Ralph	Bax	European Medicines Agency	
Gabor	Bethlendy	Parabase Genomics	
Andy	Bhattacharjee	Parabase Genomics	
Christina	Bucci-Rechtweg	Novartis Pharmaceuticals	
Laura	Butte	Critical Path Institute	
Jennifer	Canvasser	NEC Society	
Alan	Cohen	Therabron Therapeutics	
Kate	Costeloe	Queen Mary University of London	
Jon	Davis	Tufts Medical Center	Co-Chair
Tom	Diacovo	Columbia University	
Wakako	Eklund	National Association of Neonatal Nurses	
Laura	Fabbri	Chiesi Pharmaceuticals	
Christine	Gleason	University of Washington	
Wolfgang	Göpel	University of Lübeck/ VOC	
Cristal	Grogan	Preemie Parent Alliance	
Lynn	Hudson	Critical Path Institute	Co-Chair
Carole	Kenner	Council of International Neonatal Nurses	
Agnes	Klein	Health Canada	
Sam	Maldonado	Johnson and Johnson	
Laura	Martin	Graham's Foundation	
Robert	Martin	Shire	

Susan	McCune	US Food and Drug Administration	
Jeffrey	Ming	Sanofi Pharmaceuticals	
Hide	Nakamura	National Center for Child Health and Development, Tokyo	
Skip	Nelson	US Food and Drug Administration	
Karen	New	Council of International Neonatal Nurses	
Mehali	Patel	Bliss	
Ron	Portman	Novartis Pharmaceuticals	Co-Chair
Ronit	Pressler	Great Ormond Street Hospital	
Junko	Sato	Pharmaceuticals and Medical Devices Agency, Japan	
Liz	Sharpe	National Association of Neonatal Nurses	
Mary	Short	Eli Lilly	
Pamela	Simpkins	Johnson & Johnson	
Keira	Sorrells	Preemie Parent Alliance	
Janet	Soul	Harvard University	
Robin	Steinhorn	Children's National Hospital	
Linda	Storari	Chiesi Pharmaceuticals	
Charlie	Thompson	Pfizer	
Adina	Tocoian	Shire	
Mark	Turner	University of Liverpool	Co-Chair
Bob	Ward	University of Utah	
Alicia	West	Critical Path Institute	
Anne	Zajicek	National Institute of Child Health and Human Development	

Workgroup Members

INC Communications Workgroup

First Name	Surname	Organization	
Christina	Bucci-Rechtweg	Novartis Pharmaceuticals	Chair
Wakako	Eklund	National Association of Neonatal Nurses	
Jennifer	Degl	Preemie Parent Alliance	
Mary	Short	Eli Lilly	
Pamela	Simpkins	Johnson & Johnson	
Mark	Turner	University of Liverpool	

Seizures Workgroup

First Name	Surname	Organization	
Albert (AJ)	Allen	Eli Lilly	
Marilee	Allen	Johns Hopkins School of Medicine	
Stephane	Auvin	Robert Debré Hospital, Paris	
Varsha	Bhatt-Mehta	University of Michigan	
Sylvie	Benchetrit	French National Agency for Medicines and Health Products Safety/PDCO	
Geraldine	Boylan	University College Cork, Ireland	
Catherine	Chiron	Inserm, French Institute Medical Research	
Tony	Daniels	UCB	
Edress	Darsey	Pfizer	
Jon	Davis	Tufts Medical Center & INC co-director	
Scott	Denne	Indiana University, Riley Children's Hospital	
Dinah	Duarte	INFARMED	
Wakako	Eklund	National Association of Neonatal Nurses	
Fernando	Gonzalez	University of California, San Fransisco	
Pierre	Gressens	Diderot University Paris	
Cristal	Grogan	Preemie Parent Alliance	
Richard	Haas	University of California, San Diego	
Cecil	Hahn	SickKids	
Pollyanna	Hardy	Oxford University	
Norman	Hershkowitz	US Food and Drug Administration	
Kun	Jin	US Food and Drug Administration	
John	Lantos	Children's Mercy Hospital	
Neil	Marlow	University College London Hospital	
Luc	Masson	INJENO (parents of children with epilepsy)	
Jennifer	Mayberry	Graham's Foundation	
Susan	McCune	US Food and Drug Administration	
Angela	Men	US Food and Drug Administration	
Karen	New	Council of International Neonatal Nurses (COINN)	
Skip	Nelson	Office of Pediatrics, US Food and Drug Administration	
Ronit	Pressler	Great Ormond Street Hospital	Co-Chair
Ron	Portman	Novartis & INC Co-director	
Heike	Rabe	Brighton & Sussex Medical School	

Phil	Sheridan	US Food and Drug Administration	
Pamela	Simpkins	Johnson & Johnson	
Janet	Soul	Harvard University	Co-Chair
Keira	Sorrells	Preemie Parent Alliance	
Brian	Tseng	Novartis Pharmaceuticals	
Mark	Turner	University of Liverpool & INC co-director	
Alexander	Vinks	Cincinnati Children's Hospital Medical Center	
Karen	Walker	Australian College of Neonatal Nurses	
Jennifer Ann	Zimmer	Eli Lilly	
Sarah	Zohar	Inserm, French Institute Medical Research	

INC Data Workgroup

First Name	Surname	Organization	
Khosrow	Adeli	Hospital for Sick Children	
Gerri	Baer	US Food and Drug Administration	
Simin	Baygani	Eli Lilly	
Yun Sil	Chang	Samsung Medical Center in Seoul, South Korea	
Kate	Costeloe	Queen Mary University of London	Co-Chair
Jonathan	Davis	Tufts Medical Center	
Thomas	Diacovo	Columbia University Medical Center	Co-Chair
Dominique	Haumont	Saint-Pierre University Hospital	
Rosemary	Higgins	National Institute of Child Health and Human Development	
Steven	Hirschfeld	National Institute of Child Health and Human Development	
Lauren	Kelly	Hospital for Sick Children, Toronto, Canada	
Satoshi	Kusuda	Tokyo Women's Medical University	
Thierry	Lacaze	The Pediatric Network in Canada	
Kei	Lui	Australian & New Zealand Neonatal Network (ANZNN)	
Susan	McCune	US Food and Drug Administration	
Neena	Modi	Imperial College London	
Hide	Nakamura	National Center for Child Health and Development, Tokyo	
Martin	Offringa	Hospital for Sick Children, Toronto, Canada	
Michael	Padula	Children's Hospital of Philadelphia	Co-Chair
Ronald	Portman	Novartis Pharmaceuticals	
Prakesh	Shah	Canadian Neonatal Network/University of Toronto	

Catherine	Sherwin	University of Utah	
Mary	Short	Eli Lilly	
Brian	Smith	Duke University	
Roger	Soll	Vermont Oxford Network	
Marta	Terrile	Novartis Pharmaceuticals	
Charlie	Thompson	Pfizer	
Mark	Turner	University of Liverpool	

Clinical Pharmacology Workgroup

First Name	Surname	Organization	
Karel	Allegaert	University of Leuven	Co-Chair
Dina	Apele-Freimane	Riga Stradins University Hospital, Latvia	
Jacob	Aranda	University Hospital of Brooklyn	
Ronald	Ariagno	Stanford University	
Jeffrey	Barrett	Sanofi Pharmaceuticals	Co-Chair
Daniel	Benjamin	Duke University	
Edmund	Capparelli	University of California, San Diego	
Edress	Darsey	Pfizer	
Jonathan	Davis	Tufts Medical Center	
Walter	Kraft	Thomas Jefferson University	
Irja	Lutsar	University of Tartu, Estonia/PDCO	
Jeffrey	Ming	Sanofi Pharmaceuticals	
Yeruk (Lily)	Mulugeta	US Food and Drug Administration	
Min-Soo	Park	Yonsei University College of Medicine	
Ronald	Portman	Novartis Pharmaceuticals	
Randy	Prescilla	Eli Lilly/Boston's Children Hospital	
Catherine	Sherwin	University of Utah	
Ine	Skottheim Rusten	Norwegian Medicines Agency	
Adina	Tocoian	Shire	
Mark	Turner	University of Liverpool	
John	Van Den Anker	Sophia Children's Hospital (the Netherlands) and Children's National Medical Center, Washington, D.C.	
Alexander	Vinks	Cincinnati Children's Hospital Medical Center	
Kelly	Wade	Children's Hospital of Philadelphia	

Siri	Wang	Norwegian Medicines Agency	
Bob	Ward	University of Utah	Co-Chair
Anne	Zajicek	National Institute of Child Health and Human Development	

Bronchopulmonary Dysplasia (BPD) Workgroup

First Name	Surname	Organization	
Steven	Abman	University of Colorado	
Ronald	Ariagno	Stanford University	
Judy	Aschner	Montefiore	
Roberta	Ballard	University of California, San Fransisco	
Eduardo	Bancalari	Jackson Medical Center, Miami	
Dirk	Bassler	University of Zurich	
Carol	Blaisdell	National Institutes of Health	
Giuseppe	Buonocore	University of Siena, Italy	
Alan	Cohen	Therabron Therapeutics	
Jonathan	Davis	Tufts Medical Center	
Daniele	De Luca	South Paris University Hospitals	
Anthony	Durmowicz	US Food and Drug Administration	
Laura	Fabbri	Chiesi Pharmaceuticals	
Wolfgang	Göpel	University of Lübeck	Co-Chair
Anne	Greenough	King's College, London	
Ninna	Gullberg	Karolinska University Hospital/PDCO	
Helmut	Hummler	University of Ulm	
Alan	Jobe	Cincinnati Children's Hospital	
Matthew	Laughon	University of North Carolina at Chapel Hill	
Susan	McCune	US Food and Drug Administration	
Marek	Migdal	Children's Memorial Health Institute	
Michael	O'Connell	Pfizer Pharmaceuticals	
Ronald	Portman	Novartis Pharmaceuticals	
Christian	Speer	University of Wurzburg, Germany	
Robin	Steinhorn	Children's National Medical Center	Co-Chair
Linda	Storari	Chiesi Farmaceutici SpA	
Adina	Tocoian	Shire	
Mark	Turner	University of Liverpool	

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