Agenda

Public Workshop: New Methods to Predict the Immunogenicity of Therapeutic Coagulation Proteins

September 17-18, 2015
Ruth Kirschstein Auditorium, Natcher Building, NIH Campus, Bethesda MD

Sponsors:
Center for Biologics Evaluation and Research, FDA
National Heart, Lung, and Blood Institute
National Hemophilia Foundation
Plasma Protein Therapeutics Association

Steering Committee:

Zuben E. Sauna, Center for Biologics Evaluation and Research, FDA (Chair)
Donna Dimichele, National Heart, Lung, and Blood Institute, National Institutes of Health
Keith Hoots, National Heart, Lung, and Blood Institute, National Institutes of Health
Neil Frick, National Hemophilia Foundation
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Basil Golding, Center for Biologics Evaluation and Research, FDA
Estelle Russek-Cohen, Center for Biologics Evaluation and Research, FDA
Jennifer Scharpf, Center for Biologics Evaluation and Research, FDA
Glenn Pierce, World Federation of Hemophilia
Mike Soucie, Centers for Disease Control and Prevention
Johannes Oldenburg, University of Bonn
Miguel Escobar, University of Texas
Alessandro Sette, La Jolla Institute for Allergy & Immunology

Day 1

8:00 AM Registration and Continental Breakfast

8:30 AM Welcome and Introduction
Peter Marks, CBER, FDA

8:45 AM Session I
Session Chair: Basil Golding, CBER, FDA

1. Samantha Gouw, Academic Medical Center Amsterdam. Genetic determinants of immunogenicity in Hemophilia A & B (20')

2. Andrew Brooks, Rutgers University. Need for well characterized clinical samples (20')
3. Sean Stowell, Emory University. Glycobiology and immunogenicity (20')
4. Basil Golding, CBER, FDA. Q&A (15')

10:00 AM Break (20')

10:20 AM <u>Session II</u>
Session Chair: Johannes Oldenburg, University of Bonn

1. Barbara Konkle, Bloodworks Northwest. Initiatives to genotype hemophilia A patients in the U.S. (20')
2. Anne Goodeve, University of Sheffield. European genotyping initiatives and lessons learned (20')
3. Alfonso Iorio, McMaster University. Updates on results from the European Haemophilia Safety Surveillance (EUHASS) and the Canadian Hemophilia Safety Surveillance (CHESS) (20')
4. Connie Miller, Centers for Disease Control and Prevention. Genetic/Genomic studies: Importance of assays & consistent test results (20')
5. Glenn Pierce, World Federation of Hemophilia. PUPs and inhibitor testing: What's the hypothesis? (20')
6. Annaliese Hilger, European Medicines Agency (EMA). EMA workshop on registries: A report (15')
7. Johannes Oldenburg, University of Bonn. Q&A (15')

12:30 PM Lunch (Boxed Lunches Provided)

1:30 PM <u>Session III</u>
Session Chair: Shannon Meeks, Emory University

1. Yow-Ming Wang, CDER, FDA. Pharmacogenomic Genome-wide Association Studies (GWAS): The FDA perspective (20')
2. Rob Scharpf, Johns Hopkins University. Pharmacogenomic GWAS for rare diseases: A statistician's perspective (20')
3. Shannon Meeks, Emory University. Q&A(15')

2:25 PM <u>Session IV</u>
Session Chair: Debra Bensen-Kennedy, CSL Behring

1. Steven Pipe, University of Michigan. Current state of new products, review of literature on phase 3 trials (20')
2. Pedro Paz, Bayer. Predicting T-cell epitopes by integrated modeling (20')

3. Kasper Lamberth, Novo Nordisk. Preclinical predictive assessment of the immunogenicity of vatreptacog alfa (20')
4. Debra Bensen-Kennedy, CSL Behring. Q&A (20')

3:45 PM Break (30')

4:15 PM <u>Session V</u>
Session Chair: Alessandro Sette, La Jolla Institute for Allergy and Immunology

1. Dan Sikkema, GlaxoSmithKline. Evaluating markers for preclinical assessment of immunogenicity, ABIRISK (20')
2. Chen Yanover, IBM. Using predictive analytics to improve patient outcome: from data to insights (20')
3. Ira Pastan, National Cancer Institute (NCI), NIH, Deimmunizing protein molecules (20')
4. Alessandro Sette, La Jolla Institute for Allergy and Immunology Q&A (15')

5:30 PM Adjourn

<u>Day 2</u>
8:00 AM Registration and Continental Breakfast

8:30 AM Recap Day 1. Mary Clare Kimber, PPTA

8:45 AM <u>Session VI</u>
Session Chair: David Wraith, University of Bristol

1. Dawn Smilek, Immune Tolerance Network. Using new immunological paradigms in hemophilia: Lessons from the Immune Tolerance Network (20')
2. Frank Horling, Baxalta. Early Markers for immunogenicity: Antibody characteristics (20')
3. David Wraith, University of Bristol. Q&A (15')

9:40 AM Break (30')

10:10 PM <u>PANEL DISCUSSION AND Q&As</u>

PUP studies: Obtaining optimal benefits for patients from a scarce resource.
Lead: Glenn Pierce (World Federation of Hemophilia)
NIH: Donna Dimichele & Keith Hoots (NHLBI)
Regulators: Lisa Faulcon (FDA); Annaliese Hilger (EMA)
Industry: Prasad Mathew (Bayer)
Patient Advocate: Neil Frick (NHF)

10:40 AM Genetic Determinants of Immunogenicity: Is there consensus?

Co-leads: Samantha Gouw (Academic Medical Center Amsterdam) & Johannes Oldenburg (University of Bonn)
NIH: Donna Dimichele & Keith Hoots (NHLBI)
Regulator: Basil Golding (FDA)
Centers for Disease Control: Michael J Soucie
Industry: Pedro Paz (Bayer)
Patient Advocate: Katie Verb (Hemophilia Federation of America)

11:10 AM What do we do with the Genetic Information: Are pharmacogenomic GWAS studies even possible?

Co-leads: Barbara Konkle (Bloodworks Northwest) & Shannon Meeks (Emory University)
NIH: Donna Dimichele & Keith Hoots (NHLBI)
Regulator: Sarah Dorff (FDA)
Industry: Timothy Hickley (Pfizer and ABIRISK perspective)
Academia: Rob Scharpf (Johns Hopkins Univ.); Ann C Goodeve (Univ. of Sheffield)
Patient Advocate: Neil Frick (NHF)

11:40 AM Preclinical assessments of immunogenicity: Are they useful? Can they inform clinical trials?

Co-leads: Alessandro Sette (La Jolla Institute for Allergy and Immunology) & Debra Bensen-Kennedy (CSL Behring)
NIH: Donna Dimichele & Keith Hoots (NHLBI)
Regulator: Zuben E. Sauna (FDA)
Academia: Ronit Mazor (NCI)
ABIRISK: Dan Sikkema (GSK)
Industry: Kasper Lamberth (Novo Nordisk)
Patient Advocate: Neil Frick (NHF)

12:10 PM Managing the Disease: Immune Tolerance Induction, Using Genetic Information, Early Markers for Immunogenicity

Co-leads: David Wraith (University of Bristol) & Miguel Escobar (University of Texas Health Science Center)
NIH: Donna Dimichele & Keith Hoots (NHLBI)
Regulator: Lisa Faulcon (FDA)
Industry: Frank Horling (Baxalta)
Patient advocate: Neil Frick (NHF)

12:40 PM Closing remarks & end of workshop. Zuben E. Sauna (FDA)

1:00 PM Adjourn