Developing Rare Disease Regulatory Strategy Under Current Global Regulatory Statutes: A Stakeholder Discussion

Wednesday, March 28, 2018

Morning Sessions

8:00 a.m. - 8:45 a.m.	**Breakfast/Networking**
8:45 a.m. - 9:00 a.m.	**Welcome** **Debbie Hart**, President & CEO, BioNJ **Andrew Mulberg, M.D. FAAP, CPI**, Vice President, Head, Global Regulatory Affairs, Amicus Therapeutics, Inc.
9:00 a.m. - 9:20 a.m.	**Setting the Stage: Overall Challenges of Conducting Clinical Trials in the Rare Disease Population: A Case Study** **David Jacoby, M.D.**, **Ph.D**, Vice President, Head, Clinical Science, BioMarin
Panel 1: Concept and Design Considerations in Rare Disease Clinical Development Plans: Discussion of the Overall Challenges	
9:20 a.m. - 9:40 a.m.	**Significance of Natural History Studies in Trial Design** **Klaus Romero, M.D., MS, FCP**, Director, Clinical Pharmacology & Quantitative Medicine, Critical Path Institute
9:40 a.m. - 10:00 a.m.	**Using Modeling and Simulation to Optimize Study Design and Data Analysis** **Vikram Sinha, Ph.D.**, Assoc. Vice President, Quantitative Pharmacology & Pharmacometrics, Merck & Co., Inc.
10:00 a.m. - 10:35 a.m.	**Panel Discussion** ***Moderator*: Vivian Kessler, RAC**, Executive Director, Global Regulatory Affairs, Amicus Therapeutics, Inc. **Charbel Haber, Ph.D.**, Vice President, Global Regulatory Sciences, Biogen **Deborah Marsden, M.D.**, Global Medical Lead, Ultragenyx Pharmaceutical, Inc. **Nita Patel, RN**, Sr. Director, Patient & Professional Advocacy, Amicus Therapeutics, Inc. **Klaus Romero, M.D., MS, FCP**, Director, Clinical Pharmacology & Quantitative Medicine, Critical Path Institute **Vikram Sinha, Ph.D.**, Assoc. Vice President, Quantitative Pharmacology & Pharmacometrics, Merck & Co., Inc.
10:35 a.m. - 10:45 a.m.	**Break**
Panel 2: Understanding the Role of Extrapolation of Data from Varying Age Cohorts: Regulatory Requirements for Pediatric/Rare Disease Drug Development	
10:45 a.m. - 11:05 a.m.	**Understanding the Role of Extrapolation of Data from Varying Age Cohorts in Rare Diseases** **Susan McCune, M.D.**, Director, Office of Pediatric Therapeutics, Office of the Commissioner U.S. Food and Drug Administration
11:05 a.m. - 11:25 a.m.	**Global Regulatory Collaborative Initiatives: How, Why and When?** **Christina Bucci-Rechtweg, M.D.**, Global Head, Pediatric & Maternal Health Policy Drug Regulatory Affairs, Novartis Pharmaceuticals
11:25 a.m. - 12:00 p.m.	**Panel Discussion** ***Moderator*: John Spaltro, Ph.D.**, Director, Global Regulatory Affairs, Amicus Therapeutics, Inc. **Christina Bucci-Rechtweg, M.D.**, Global Head, Pediatric & Maternal Health Policy Drug Regulatory Affairs, Novartis Pharmaceuticals **Susan McCune, M.D.**, Director, Office of Pediatric Therapeutics, Office of the Commissioner U.S. Food and Drug Administration **Scott McGoohan, J.D.**, Director, U.S. Regulatory Policy & Intelligence, Vertex Pharmaceuticals **Lily (Yeruk) Mulugeta, PharmD**, Clinical Reviewer, Division of Pediatrics, CDER U.S. Food and Drug Administration **Robert "Skip" Nelson, M.D., Ph.D.**, Sr. Director, Pediatric Drug Development, Child Health Innovation Leadership Dept. (CHILD), Johnson & Johnson **Ben Stockham**, Head, International Regulatory Affairs, Amicus Therapeutics, Inc.

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Afternoon Sessions

12:00 p.m. - 1:00 p.m.	**Lunch**
1:00 p.m. - 1:30 p.m.	**Novel Statistical Design Considerations in Rare Disease Drug Development** **Qing Liu, Ph.D.**, Sr. Director, Biostatistics and Data Management, Amicus Therapeutics, Inc.
1:30 p.m. - 1:45 p.m.	**Break**
Panel 3: Pre-Competitive Interactions and Concepts Development (Select 1 breakout session)	
1:45 p.m. - 3:00 p.m.	**Breakout 1: Registry Development** ***Moderators***: **Joseph Giuliano**, Executive Director, Global Medical Operations and Patient Registry, Amicus Therapeutics, Inc. **Sheela Sitaraman, Ph.D., PMP**, Sr. Director, Program Management, Amicus Therapeutics, Inc. ***Panel***: **Grace Pavlath, M.D., Ph.D**., Sr. Vice President, Scientific Program Director Muscular Dystrophy Association **Klaus Romero, M.D., MS, FCP**, Director, Clinical Pharmacology & Quantitative Medicine Critical Path Institute
1:45 p.m. - 3:00 p.m.	**Breakout 2: Dose Selection and Dose Ranging** ***Moderators***: **Franklin K. Johnson, Ph.D.**, Sr. Director, Clinical Pharmacology, Amicus Therapeutics, Inc. **Vikram Sinha, Ph.D.**, Assoc. Vice President, Quantitative Pharmacology & Pharmacometrics Merck & Co., Inc. ***Panel:*** **Karim Azer, Ph.D.**, Head, Quantitative Systems Pharmacology, Sanofi U.S. **Mark Gastonguay, Ph.D., FISoP**, CEO, Metrum Research Group, LLC **Lily (Yeruk) Mulugeta, PharmD**, Clinical Reviewer, Division of Pediatrics, CDER U.S. Food and Drug Administration
1:45 p.m. - 3:00 p.m.	**Breakout 3: Role of Extrapolation** ***Moderator:*** **Susan McCune, M.D.**, Director, Office of Pediatric Therapeutics, Office of the Commissioner U.S. Food and Drug Administration ***Panel***: **Charbel Haber, Ph.D.**, Vice President, Global Regulatory Sciences, Biogen **David Jacoby, M.D., Ph.D**, Vice President, Head, Clinical Science, BioMarin **Andrew Mulberg, M.D. FAAP, CPI**, Vice President, Head, Global Regulatory Affairs Amicus Therapeutics, Inc. **Scott McGoohan, J.D.**, Director, U.S. Regulatory Policy & Intelligence, Vertex **Robert "Skip" Nelson, M.D., Ph.D.**, Sr. Director, Pediatric Drug Development, Child Health Innovation Leadership Dept. (CHILD), Johnson & Johnson
3:00 p.m. - 3:15 p.m.	**Break**
3:15 p.m. - 4:00 p.m.	**Panel Summary and Next Steps** All Sections Leaders
4:00 p.m. - 4:15 p.m.	**Closing Remarks** **Andrew Mulberg, M.D. FAAP, CPI**, Vice President, Head, Global Regulatory Affairs Amicus Therapeutics Inc. **Debbie Hart**, President & CEO, BioNJ