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Public Health

 **EASL**
European Association
for the Study of the Liver



**Forum for
Collaborative HIV Research**

Liver Forum 5
November 10, 2016
The Westin Copley Place
Boston, MA

1:30 PM Welcome Coffee Reception & Check-in

2:00 PM Session I: Introduction & Updates

Moderators:

Veronica Miller, *Forum for Collaborative HIV Research*
 Arun Sanyal, *Virginia Commonwealth University Medical Center*
 David Shapiro, *Intercept Pharmaceuticals*

2:00 PM Liver Forum Updates

Presenters:

Arun Sanyal, *Virginia Commonwealth University Medical Center*
 Veronica Miller, *Forum for Collaborative HIV Research*

2:15 PM Regulatory Updates

Presenters:

Lara Dimick-Santos, *U.S. Food and Drug Administration*
 Elmer Schabel, *Bundesinstitut für Arzneimittel und Medizinprodukte*

2:45 PM Recognition for Outstanding Service

Presenters:

Arun Sanyal, *Virginia Commonwealth University Medical Center*
 David Shapiro, *Intercept Pharmaceuticals*

Recipient:

Captain Anissa Davis-Williams, *U.S. Food and Drug Administration*

2:50 PM Session II: Focus on Biomarkers

Moderators:

Veronica Miller, *Forum for Collaborative HIV Research*
 Quentin Anstee, *Newcastle University Medical School*

2:50 PM FDA Biomarker Qualification in Drug Development Under IND or NDA/BLA

Presenter:

Shashi Amur, *U.S. Food and Drug Administration*

3:10 PM FNIH: NASH Biomarker Consortium

Presenter:

Roberto Calle, *on behalf of FNIH*

3:20 PM Liver Forum - FNIH Collaborations

Presenter:

Arun Sanyal, *co-chair FNIH Biomarker Consortium*

3:30 PM IMI: Accelerated Drug Portal

Presenter:

Julia Brosnan, *on behalf of IMI*

3:35 PM **Session III: Definitions WG Update**

Moderators:

David Shapiro, *Intercept Pharmaceuticals*
Stephen Harrison, *University of Oxford*

3:35 PM Beyond Baseline Definitions: Scientific Agenda Moving Forward

Presenter:

Sophie Megnien, *Genfit Corp*

Discussants:

Pierre Bedossa, *University of Paris Diderot*
Vlad Ratziu, *Hôpital Pitié Salpêtrière et Université Pierre et Marie Curie*
Brent Tetri, *Saint Louis University School of Medicine*
Saul Karpen, *Emory University School of Medicine*
Jeff Schwimmer, *University of California, San Diego School of Medicine*
Vincent Wong, *The Chinese University of Hong Kong*

4:15 PM **Coffee Reception**

4:45 PM **Session IV: Pediatric Updates**

Moderators:

Joel Lavine, *Columbia University Medical Center*
Richard Torstenson, *Novo Nordisk*

4:45 PM Regulatory Updates

Presenters:

Elmer Schabel, *Bundesinstitut für Arzneimittel und Medizinprodukte*
Ruby Mehta, *U.S. Food and Drug Administration*

5:00 PM Status of Pediatric NASH Research

Presenters and Panelists

Miriam Vos, *Emory University School of Medicine*
Jeff Schwimmer, *University of California, San Diego School of Medicine*
Ruby Mehta, *U.S. Food and Drug Administration*
Rajarshi Banerjee, *Perspectum Diagnostics*

5:40 PM

Session V: Mechanism of Action and Surrogate Endpoints

Moderators:

David Shapiro, *Intercept Pharmaceuticals*
Arun Sanyal, *Virginia Commonwealth University Medical Center*

5:40 PM

Linking Mechanism of Action to Endpoint Selection in NASH Trial

Panel:

Laurent Fischer, *Tobira Therapeutics, Inc.*
Rob Myers, *Gilead Sciences, Inc.*
Scott Friedman, *Icahn School of Medicine at Mount Sinai*
Anna Mae Diehl, *Duke University Medical Center*
Detlef Schuppan, *Mainz University Medical Center*
Eileen Navarro, *U.S. Food and Drug Administration*

6:20 PM

Session VI: New WG Prioritization

Moderators:

Veronica Miller, *Forum for Collaborative HIV Research*
Arun Sanyal, *Virginia Commonwealth University Medical Center*

6:20 PM

Possible New Working Groups:

1. Efficiency in Trial Recruitment
2. Framework for Placebo Arm Cohort
3. Adaptive trial design and new analytic approaches
4. Biomarker Surrogate Endpoints
5. DILI/NASH
7. HCC and Other Disease States
6. Other?

Discussants:

Claudia Filozof, *Covance*
Donna Cryer, *Global Liver Institute*
Peter Traber, *Galectin Therapeutics, Inc.*
Robert Arch, *Takeda Pharmaceuticals International, Inc.*
Sudha Shankar, *FNIH*
Claude Cohen-Bacrie, *SuperSonic Imagine*
Claude Sirlin, *University of California, San Diego*
Erwin de Buijzer, *Humedics GmbH*
Arie Regev, *Eli Lilly and Company*
Joanne Imperial, *FibroGen, Inc.*

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**Forum for
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Session I: Welcome, Updates and Award



Forum's Operating Principle

*Facilitating collaborative research in drug
development and health policy*

“Once new drug candidates and therapeutic strategies are identified, their efficient, safe development is in the best interest of all stakeholders, most of all, the patients”



Impact on Drug Development

*Facilitating collaborative research in drug
development and health policy*

- **Increase**
 - Clarity
 - Efficiency
 - Collaboration
 - Innovation
- **Decrease**
 - Uncertainty
 - Redundancy
 - Development time
 - Risk



*Facilitating collaborative research in drug
development and health policy*

Win-Win

- Accelerate drug development not by lowering standards, but by increasing efficiency through collaboration



Operating Principles (see handouts)

- Equal voice
- Co-ownership of process
- Open discussion and deliberation
- What's said at the LF stays at the LF
- Industry participation:
 - Not contingent on financial support
 - LF assemblies: 2 max/company
 - More may participate in Working Groups
 - Scientists and clinical researchers
 - Not for marketing and commercial branches



Operating Principles (see handouts)

- Equal voice
- Co-ownership of process
- Open discussion and deliberation
- What's said at the LF stays at the LF
- Industry participation:
 - Not contingent on support
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 - Scientists and clinical researchers
 - Not for marketing and commercial branches

Non-Competitive, Safe Zone



*Facilitating collaborative research in drug
development and health policy*

Achievements

- Space for informal exchange of regulatory perspectives
 - EU, US
 - (other agencies?)
- New collaborations and networks among Liver Forum members
- Increasing regulatory clarity in the field of NASH



*Facilitating collaborative research in drug
development and health policy*

Tomorrow's Leadership

- Foster professional development
 - Junior faculty
 - Fellows
 - Interns
- Principles of collaborative drug development and regulatory science
 - Trans-Atlantic
 - Worldwide



LF4 → LF5

- LF1: November 2014
- Manuscripts:
 - Disease Case Definition, Baseline – Submission ready
 - Standardization of Baseline Parameters – Final agency review
 - Pediatric Issue – Preparing for final agency review
 - Transatlantic consensus
 - Living documents



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Forum “Fellows”

- **Mohammad Shadab Siddiqui**
 - Arun Sanyal, Virginia Commonwealth University
- **Yuval Patel**
 - Andrew Muir, Duke University
- **Amanda Cheung**
 - Brent Tetri, SSM Health Saint Louis University Hospital



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development and health policy*

New Initiatives -1

- NASH Biomarker Workshop
 - In collaboration with FNIH NASH Biomarker Consortium and EME
 - May 5-6, 2017
 - ◉ Washington DC
 - Abstract driven workshop



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New Initiatives - 2

- Focused workshops (LF5 → LF6)
 - 1. Adaptive Trial Design and New Analytic Approaches
 - 2. Pediatric natural history cohort – challenges, strategies and innovation



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development and health policy

Liver Forum Steering Committee

- Co-chairs
 - David Shapiro, *Intercept Pharmaceuticals*
 - Arun Sanyal, *VCU*
- FDA
 - Lara Dimick, *CDER/DGIEP*
 - Ruby Mehta, *CDER/DGIEP*
 - Chris Leptak, *CDER/OND*
- EMA
 - Elmer Schabel, *BfArM*
- Community/Patients
 - William Baldyga, *Patient Rep*
 - Donna Cryer, *Global Liver Institute*
- Academia
 - Scott Friedman, *Mount Sinai*
 - Miriam Vos, *Emory University*
 - Vlad Ratziu, *Université Pierre et Marie Curie Hospital Pitie Salpetriere*
 - Detlef Schuppan, *Mainz Uni Med Center/BIDMC*
- Industry
 - Gary Burgess, *Vectura Limited*
 - Laurent Fischer, *Tobira Therapeutics, Inc.*
 - Sophie Megnien, *Genfit*
 - Rob Myers, *Gilead Sciences, Inc.*
- Societies
 - Stephen Harrison, *AASLD*
 - Laurent Castera, *EASL*
 - Tom Hemming Karlsen, *EASL*



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 - Laurent Castera, *EASL*
 - Tom Hemming Karlsen, *EASL*



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Thanks to outgoing SC members

- Helena Brett-Smith (BMS)
- Carol Brosgart (UCSF)
- Joel Lavine (Columbia)
- Massimo Pinzani (University College London)

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Industry Membership and Sponsorship Update

Veronica Miller, PhD

veronicam@berkeley.edu

www.hivforum.org



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Industry Members

Total Industry Members - 96 organizations

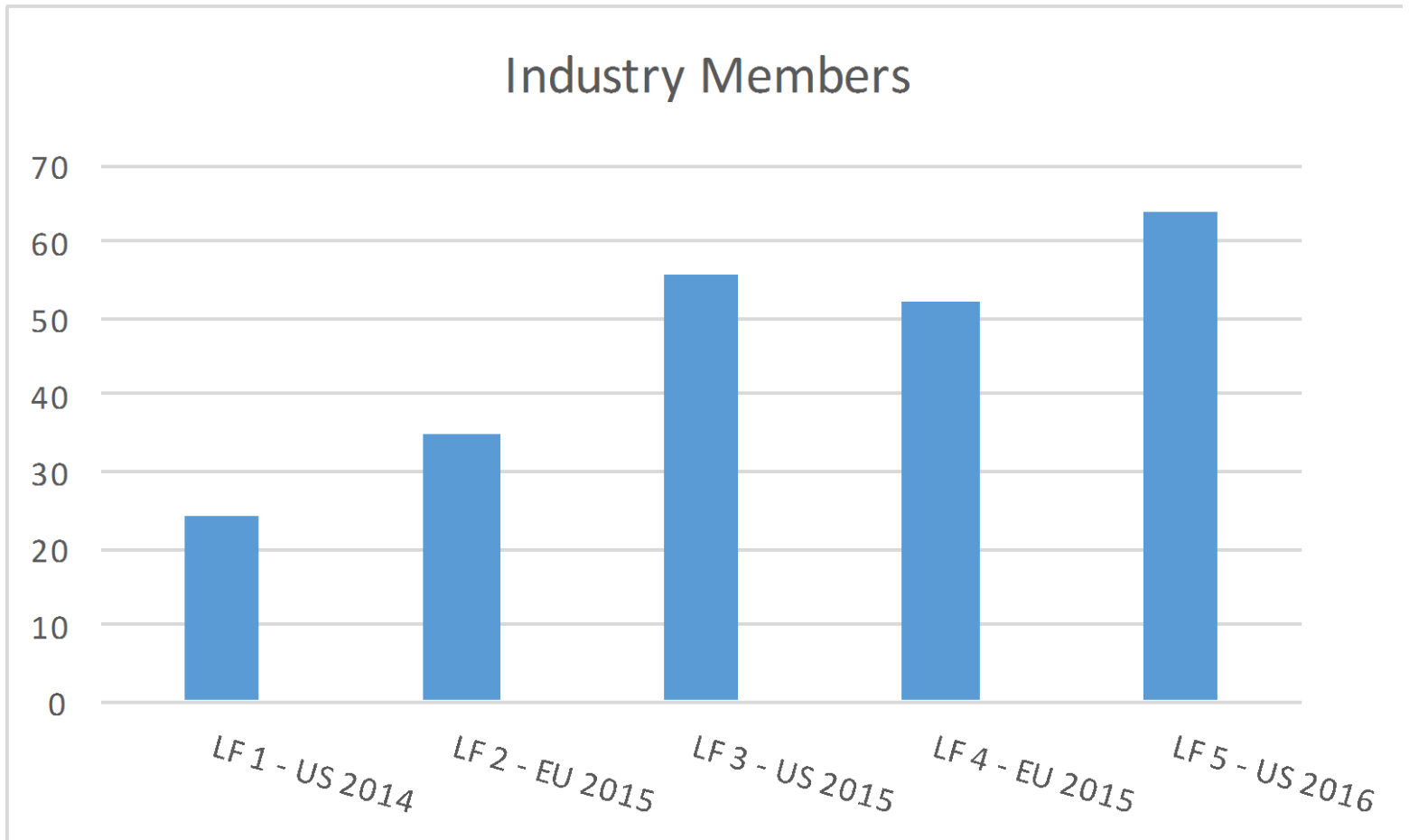
- 37 Pharma
- 36 Biopharmaceuticals
- 17 Diagnostic
- 6 Clinical Research Organizations (CRO)

Inclusive of 8 total start-ups



Participating Organizations by Year

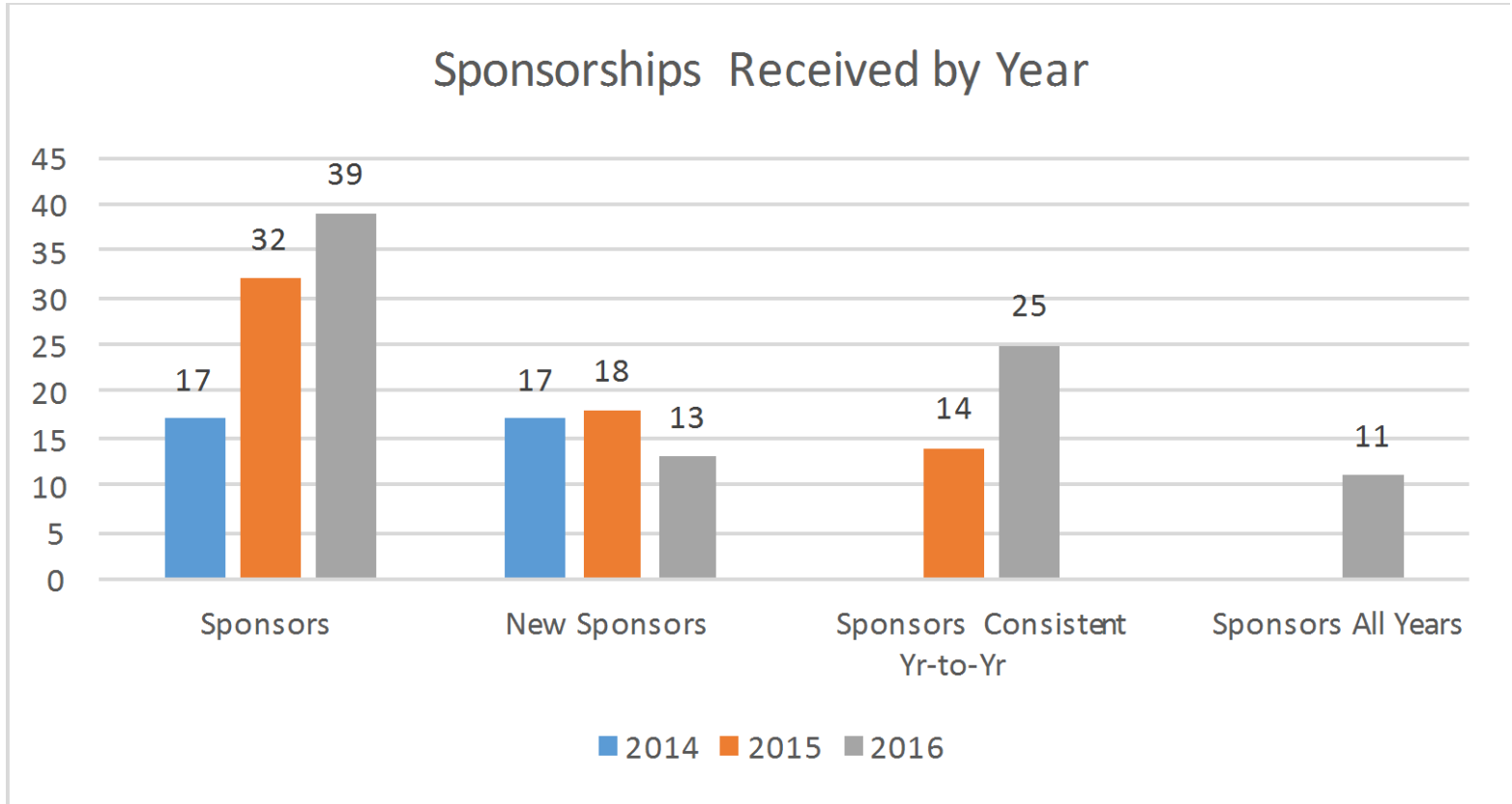
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Industry Sponsorships

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Financial Sponsors

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 Boehringer
Ingelheim



Bristol-Myers Squibb

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John Joseph Sninsky &
Susan Bardsley Sninsky





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Many Thanks

- Forum Staff
 - Malene Cobourne
 - Ben Hauschild
 - Pedro Goicochea
 - Katie Greene
 - Jeffrey Kaminski
 - Brenda Rodriguez
 - Ken Taymor
- Prism Event Management
 - Paula Blay
 - Graham Hill
 - Ash Lawson
 - Mairead O'Reilly