Welcome to the CMC Strategy Forum Europe 2019

The 13th annual CMC Strategy Forum Europe, organized by CASSS, will explore many critical topics focused on improving the quality in development and manufacturing of biopharmaceutical products. A series of plenary sessions and workshops led by experts from global regulatory agencies, academia and industry seek to explore emerging aspects of CMC technology and regulation in areas where existing modalities and systems are undergoing change. Topics will include: Regulatory Updates from Around the Globe; ICH Q12 and Challenges of Post-approval Changes; CMC Challenges in Accelerated Drug Approval Pathways including PRIME and Breakthrough; ICH Q14 / Q2 – Trends and Concepts for QbD for Analytics and Lifecycle Management of Analytical Methods; Continuous Manufacturing for Biologics; and Personalised Medicine and Manufacturing 4.0.

The EBE session will present updates on the following concept papers: Raw Materials; Medicinal Product Incorporating a Drug Delivery Device Component: An Industry Perspective on Developing an Efficient “End-to-End” Control Strategy; and Polysorbates, as well as the workshop topic: CRISPR-Cas9.

The CMC Strategy Forum is designed to maximize dialog between participants. Presentations are relatively short and focused and set the agenda for the panel discussions to engage all the participants who have experience and expertise to share. It should be important for you to attend this event as we come together to discuss important issues on how to ensure product safety and efficacy for the patients we serve.

We would like to thank the speakers and panel members who are giving generously of their time and resources, and to you, for your attendance. We acknowledge the generosity of our program partners: Amgen Inc.; AstraZeneca; Biogen, Bristol-Myers Squibb Company; Eli Lilly and Company; F. Hoffmann-La Roche Ltd.; IPSEN Biopharm Ltd.; MSD; Novo Nordisk A/S and Pfizer, Inc. We are grateful for the expert management from CASSS and the audio-visual expertise of Michael Johnston from MJ Audio-Visual Productions. Their experience and guidance in the preparation of this Forum has been invaluable.
ACKNOWLEDGEMENTS

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## Monday, 13 May 2019

### EBE-European Biopharmaceutical Enterprises Satellite Session

<table>
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<th>Time</th>
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| 06:30 – 10:30 | **Breakfast** in the Mosaic Restaurant
*(Breakfast is included in the CMC Strategy Forum group sleeping room rate; other attendees / guests can pay individually for breakfast if they are not included in the group room rate)* |
| 07:30 – 12:00 | **Registration** in the Giralda Foyer                                                                |
| 08:30 – 08:45 | **Welcome and Introduction to the European Biopharmaceutical Enterprises (EBE) Ongoing Activities and Initiatives** in Giralda I-II Ballroom
Markus Goese, *F. Hoffmann-La Roche Ltd.*, EBE Biomanufacturing Working Group Chair                              |
| 08:45 – 09:00 | **Concept Paper 2019 Updates** In Giralda I-II Ballroom  
**Session Chairs:** Karoline Bechtold-Peters, *Novartis Pharma AG* and Fionnuala O’Driscoll, *Eli Lilly Kinsale Limited* |
| 09:00 – 09:15 | **Medicinal Product Incorporating a Drug Delivery Device Component: An Industry Perspective on Developing an Efficient ‘End-to-End’ Control Strategy**
Carolyn Gordon, *AstraZeneca, United Kingdom*                                                               |
| 09:15 – 09:30 | **Polysorbates**
Karoline Bechtold-Peters, *Novartis Pharma AG, Switzerland* (on behalf of EBE Industry Consortium)          |
| 09:30 – 10:00 | **Panel Discussion – Questions and Answers**
Karoline Bechtold-Peters, *Novartis Pharma AG, Switzerland* (on behalf of EBE Industry Consortium)  
Annick Gervais, *UCB SA, Belgium*  
Carolyn Gordon, *AstraZeneca, United Kingdom*                                                              |
<p>| 10:00 – 10:30 | <strong>Networking Break</strong> in the Giralda Foyer                                      |</p>
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<tr>
<th>Time</th>
<th>Session Title</th>
<th>Speaker(s)</th>
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<tr>
<td>10:30 – 10:50</td>
<td>What is CRISPR-Cas9 and Using CRISPR-based Technologies to Modulate Endogenous Gene Expression Levels</td>
<td>Gerald Klanert, Austrian Centre of Industrial Biotechnology, Austria</td>
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<tr>
<td>10:50 – 11:10</td>
<td>Replicating Human Disease in Rodents: The Good and the Bad of CRISPR/Cas9 Genome Editing</td>
<td>Guillaume Pavlovic, Phenomin, France</td>
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<td>11:10 – 11:30</td>
<td>Regulatory Considerations for Medicinal Products Consisting of or Manufactured by Genome Editing Tools</td>
<td>Marcos Timón, AEMPS-Spanish Agency of Medicines and Medical Devices, Spain</td>
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<tr>
<td>11:30 – 12:00</td>
<td>Panel Discussion – Questions and Answers</td>
<td>Gerald Klanert, Austrian Centre of Industrial Biotechnology, Austria</td>
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<td>Guillaume Pavlovic, Phenomin, France</td>
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<td>Marcos Timón, AEMPS-Spanish Agency of Medicines and Medical Devices, Spain</td>
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<tr>
<td>12:00 – 12:15</td>
<td>Concluding Remarks</td>
<td>Véronique Debaut, EBE-European Biopharmaceutical Enterprises, Belgium</td>
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Monday, 13 May continued…

CMC Strategy Forum Europe 2019
Scientific Program Summary

12:15 – 13:45 Buffet Lunch in the Mosaic Restaurant

13:15 – 17:30 Registration in the Giralda Foyer

13:45 – 14:00 CASSS Welcome and Introductory Comments in Giralda I-II Ballroom
Wassim Nashabeh, F. Hoffmann-La Roche Ltd., USA

Introduction / Welcome to the 13th European CMC Strategy Forum
Ronald Imhoff, Janssen Biologics BV, Netherlands

Regulatory Updates from Around the Globe
Plenary Session in Giralda I-II Ballroom
Session Chairs: Ronald Imhoff, Janssen Biologics BV, Ilona Reischl, BASG-Federal Office for Safety in Health Care, and Martijn van der Plas, Medical Evaluations Board (MEB)

14:00 – 14:25 How Regulators Can Promote Innovation
César Hernández, AEMPS-Spanish Agency of Medicines and Medical Devices, Spain

14:25 – 14:50 US FDA Update: Recent Trends in the Regulation of Biopharmaceuticals
Emanuela Lacana, CDER, FDA, USA

14:50 – 15:15 Recent Trend of in the Regulation of Biological Products in China
Suyuan Cheng, CDE / NMPA-National Medicinal Products Administration, China

15:15 – 15:40 Drug-Device Combination Products: Quality Considerations
Nick Lee, HPRA-Health Products Regulatory Authority, Ireland

15:45 – 16:15 Networking Break in the Giralda Foyer

16:15 – 17:30 Panel Discussion – Questions and Answers
Esra’a Abdelrahman S. Alzubi, JFDA-Jordan Food and Drug Administration, Jordan
Suyuan Cheng, CDE / NMPA-National Medicinal Products Administration, China
César Hernández, AEMPS-Spanish Agency of Medicines and Medical Devices, Spain
Rita Yazbeck Karam, Ministry of Public Health, Lebanon
Emanuela Lacana, CDER, FDA, USA
Nick Lee, HPRA-Health Products Regulatory Authority, Ireland
Monday, 13 May continued…

17:30 – 17:45  Mini-break

| Continuous Manufacturing for Biologics  
| Workshop Session One in Giralda I-II Ballroom  
| **Session Chairs:** Robin Levis, **CBER, FDA,** and Bridgett O'Shea, **Pfizer, Inc.** |

17:45 – 17:50  Introduction

17:50 – 18:15  The EMA Regulatory Perspective on Continuous Manufacturing for Biologicals
Veronika Jekerle, European Medicines Agency (EMA), Netherlands

18:15 – 18:40  A Forum Report for Continuous Manufacturing of Biologics
Andrew Chang, Novo Nordisk Inc., USA

18:40 – 19:05  Challenges during the Development of a High Cell Density Continuous (HCD) Upstream Process
Kathleen Barrette, Sanofi, France

19:05 – 19:30  Leveraging Continuous Manufacturing to Reduce Facility Footprint
Thomas Urbig, Amgen Research Munich GMbH, Germany

19:30 – 20:30  Panel Discussion – Questions and Answers
Kathleen Barrette, Sanofi, France
Andrew Chang, Novo Nordisk Inc., USA
Veronika Jekerle, European Medicines Agency (EMA), Netherlands
Nick Lee, HPRA-Health Products Regulatory Authority, Ireland
Thomas Urbig, Amgen Research Munich, Germany

20:30  Adjourn Day One
Tuesday, 14 May 2019

06:30 – 10:30  Breakfast in the Mosaic Restaurant
     (Breakfast is included in the CMC Strategy Forum group sleeping room rate; other attendees / guests can pay individually for breakfast if they are not included in the group room rate)

08:00 – 17:00  Registration in the Giralda Foyer

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<th>CM C Challenges in Accelerated Drug Approval Pathways including PRIME and Breakthrough Workshop Session Two in Giralda I-II Ballroom</th>
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<td><strong>Session Chairs:</strong> Michael Abernathy, Amgen Inc., Sandra Auguste-Bowler, H. Lundbeck A/S, and Thomas Schreitmüller, F. Hoffmann-La Roche Ltd.</td>
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09:00 – 09:05  Introduction

09:05 – 09:50  Regulatory Challenges in Early Access Approaches – Reflections from an EMA/FDA Workshop
     Marcel Hoefnagel, CBG-MEB, Medical Evaluations Board, Netherlands
     Veronika Jekerle, EMA-European Medicines Agency, Netherlands
     Mats Welin, MPA-Medical Products Agency, Sweden

09:50 – 10:15  Accelerated Development as the “New Normal”: Process Validation Considerations
     Lisa deCardenas, Genentech, a Member of the Roche Group, USA

     Andrew Lennard, Amgen Limited, United Kingdom

10:45 – 11:15  Networking Break in the Giralda Foyer

11:15 – 12:15  Panel Discussion – Questions and Answers
     Lisa deCardenas, Genentech, a Member of the Roche Group, USA
     Chana Fuchs, CDER, FDA, USA
     Marcel Hoefnagel, Medical Evaluations Board, Netherlands
     Ronald Imhoff, Janssen BV, Netherlands
     Veronika Jekerle, European Medicines Agency (EMA), Netherlands
     Andrew Lennard, Amgen Limited, United Kingdom
     Mats Welin, Medical Products Agency, Sweden

12:15 – 13:45  Buffet Lunch in the Mosaic Restaurant
Tuesday, 14 May continued…

ICH Q14 / Q2 - Trends and Concepts for QbD for Analytics and Lifecycle Management of Analytical Methods

**Workshop Session Three** in Giralda I-II Ballroom

**Session Chairs:** Emmanuelle Charton, *EDQM, Council of Europe*, Alistair Kippen, *IPSEN Biopharm Limited* and Heli Suila, *Finnish Medicines Agency*

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| 13:50 – 14:15 | QbD in Analytics – Opportunities for Method Development and Life Cycle Management  
                Christof Finkler, *F. Hoffmann-La Roche Ltd., Switzerland* |
| 14:15 – 14:40 | How Can Revising ICH Q2 Change the Conversation about Analytical Method Validation?  
                Mary Beth Pelletier, *Biogen, USA* |
| 14:40 – 15:05 | ICH Q2/Q14: Reflections for Vaccines  
                Cristiana Campa, *GlaxoSmithKline S.p.A, Italy* |
| 15:05 – 15:30 | Reflections from an Authority Perspective  
                Tone Agasøster, *Norwegian Medicines Agency, Norway* |
| 15:30 – 16:00 | Networking Break in the Giralda Foyer                                    |
| 16:00 – 17:00 | Panel Discussion – Questions and Answers  
                Tone Agasøster, *Norwegian Medicines Agency, Norway*  
                Cristiana Campa, *GlaxoSmithKline, Italy*  
                Christof Finkler, *F. Hoffmann-La Roche Ltd., Switzerland*  
                Nanna Kruse, *Danish Medicines Agency, Denmark*  
                Robin Levis, *CBER, FDA, USA*  
                Mary Beth Pelletier, *Biogen, USA*  
                Miao Xu, *NIFDC / NMPA-National Medicinal Products Administration, China* |
| 17:00       | Adjourn Day Two                                                          |
| 18:30 – 21:00 | Off Property Networking Reception                                        |
Wednesday, 15 May 2019

06:30 – 08:45 Breakfast in the Mosaic Restaurant
(Breakfast is included in the CMC Strategy Forum group sleeping room rate; other attendees / guests can pay individually for breakfast if they are not included in the group room rate)

08:30 – 17:00 Registration in the Giralta Foyer

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<th>Workshop Session Four in Giralta I-II Ballroom</th>
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<td>Session Chairs: Chana Fuchs, CDER, FDA, Kowid Ho, F. Hoffmann-La Roche Ltd., and Tara Sanderson, UCB Pharma Ltd.</td>
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09:00 – 09:05 Introduction

09:05 – 09:30 ICH Q12 Challenges of Post-approval Changes
Nanna Kruse, Danish Medicines Agency, Denmark

09:30 – 09:50 The Complexity of Post approval Changes for Legacy Products
Carine Dortu, UCB Biopharma sprl, Belgium

09:50 – 10:15 Challenges of Q12 and Post-Approval CMC Changes in China
Jinzhong Xiang, CDE / NMPA-National Medicinal Products Administration, China

10:15 – 10:45 Networking Break in the Giralta Foyer

10:45 – 11:05 Putting ICH Q12 into Action: Established Conditions for Original MAAs and Established Products
Vandana Chauhan, F. Hoffmann-La Roche Ltd., Switzerland

11:05 – 11:25 The Opportunities and Challenges for the Implementation of ICH Q12 from an AstraZeneca Perspective
Stuart Finnie, AstraZeneca, United Kingdom

11:25 – 12:25 Panel Discussion – Questions and Answers
Vandana Chauhan, Genentech, a Member of the Roche Group, USA
Carine Dortu, UCB Pharma S.A., Belgium
Stuart Finnie, AstraZeneca, United Kingdom
Nanna Kruse, Danish Medicines Agency, Denmark
Emanuela Lacana, CDER, FDA, USA
Jinzhong Xiang, CDE / NMPA-National Medicinal Products Administration, China

12:30 – 13:45 Buffet Lunch in the Mosaic Restaurant
**Personalised Medicine and Manufacturing 4.0**  
**Workshop Session Five** in Giralta I-II Ballroom  
**Session Chairs:** Seán Barry, HPRA-Health Products Regulatory Authority, Ralf Gleixner, Ares Trading SA, An affiliate of Merck Serono and Brendan Hughes, Bristol-Myers Squibb Company

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<td>13:45 – 13:50</td>
<td><strong>Introduction</strong></td>
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| 13:50 – 14:15 | **Pharma 4.0: Departure into a New Dimension**  
Lothar Hartmann, PHACT GmbH, Germany |
| 14:15 – 14:40 | **Monitoring the Viability and Metabolic State of Cells during a Bioprocess**  
Michael Butler, NIBRT-National Institute for Bioprocessing Research and Training, Ireland |
| 14:40 – 15:05 | **Next Generation Manufacturing Technologies to Enable the Delivery of High Mix, Low Volume Drug Products and the Journey to Personalized Medicine**  
Mark Quinlan, Amgen Inc., USA |
| 15:05 – 15:30 | **Are We There Yet? Roadmap and Execution of Advanced Data Analytics to Generate Actionable Insights from Biopharma Manufacturing**  
Syama Adhibhatta, Bristol-Myers Squibb Company, USA |
| 15:30 – 16:00 | **Networking Break** in the Giralta Foyer |
| 16:00 – 17:00 | **Panel Discussion – Questions and Answers**  
Syama Adhibhatta, Bristol-Myers Squibb Company, USA  
Brigitte Brake, BfArM, Germany  
Michael Butler, NIBRT-National Institute for Bioprocessing Research and Training, Ireland  
Lothar Hartmann, PHACT GmbH, Germany  
Martin Nemec, Health Canada, Canada  
Mark Quinlan, Amgen Inc., USA |
| 17:00 – 17:15 | **Closing Remarks and Invitation to CMC Strategy Forum Europe 2020**  
Sandra Auguste-Bowler, Lundbeck A/S |
| 17:15 | **Adjournment** |