# 22nd Annual John Goldman E-Conference on Chronic Myeloid Leukemia: Biology and Therapy

**October 1-4, 2020**

**Chairs:** J. Cortes, T.P. Hughes, D.S. Krause  
**Co-Organizers:** R. Bhatia, T. Brümmendorf, M. Copland, M. Deininger, O. Hantschel, F-X. Mahon, D. Perrotti, J. Radich, D. Réa  
**Advisory Committee:** J. Apperley, S. Branford, C. Gambacorti-Passerini, F. Guilhot, R. Hehlmann, P. Laneuville, G. Saglio, C. Schiffer, S. Soverini, P. Valent, R. Van Etten

*This program is presented according to Central European Summer Time (CEST). Please check the corresponding hours in your time zone.*

---

### Thursday, October 1, 2020

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Title</th>
<th>Presenter(s)</th>
</tr>
</thead>
<tbody>
<tr>
<td>14:00-14:10</td>
<td>WELCOME</td>
<td></td>
<td>Jorge Cortes (Augusta)</td>
</tr>
<tr>
<td>14:10-14:20</td>
<td>iCMLf - A REVIEW</td>
<td></td>
<td>Tim P. Hughes (Adelaide)</td>
</tr>
<tr>
<td>14:20-14:53</td>
<td>SPECIAL INTRODUCTORY LECTURE</td>
<td></td>
<td>Jerry Radich (Seattle)</td>
</tr>
<tr>
<td>14:20-14:23</td>
<td>Introduction</td>
<td></td>
<td></td>
</tr>
<tr>
<td>14:23-14:43</td>
<td>Finding relapse-fated cells at diagnosis and targeting relapse before it evolves</td>
<td>John Dick (Toronto)</td>
<td></td>
</tr>
<tr>
<td>14:43-14:53</td>
<td>Discussion</td>
<td></td>
<td></td>
</tr>
<tr>
<td>14:53-16:40</td>
<td>SCIENTIFIC SESSION 1: TOP SCORING ABSTRACTS 2020</td>
<td></td>
<td>Brian Huntly (Cambridge), Francois -Xavier Mahon (Bordeaux)</td>
</tr>
<tr>
<td>14:53-14:56</td>
<td>Introduction</td>
<td></td>
<td></td>
</tr>
<tr>
<td>14:56-15:08</td>
<td>Targeted RNA-Based Gene Sequencing Identifies Mutated Blood Cancer-Linked Genes at Diagnosis of CML That Are Associated with Treatment Failure</td>
<td>Susan Branford (Adelaide)</td>
<td></td>
</tr>
<tr>
<td>15:08-15:20</td>
<td>Tgfβr - SMAD3 Signaling Protects CML and Other Myeloid Malignancies from Synthetic Lethality Triggered By PARP Inhibitor in Bone Marrow Microenvironment</td>
<td>Bac Viet Le (Warsaw/Philadelphia)</td>
<td></td>
</tr>
<tr>
<td>15:20-15:32</td>
<td>Interim Analysis from the Optic Trial, a Dose-Ranging Study of 3 Starting Doses of Ponatinib</td>
<td>Jorge Cortes (Augusta)</td>
<td></td>
</tr>
<tr>
<td>15:32-15:44</td>
<td>Deceiving and Drugging BCR-ABL1 Compound Mutants: Pairing a Conformational Influencer with Ascinib or an Ascinib Proteolysis-Targeting Chimera (PROTAC)</td>
<td>Thomas O’Hare (Salt Lake City)</td>
<td></td>
</tr>
</tbody>
</table>
15:44-15:56 Therapeutic Inhibition of Malignant Fcγrllb-Induced BTK Activation Targets Leukemic Stem Cells in Chronic Myeloid Leukemia Mirle Schemionek (Aachen)

15:56-16:08 Molecular Status 36 Months after TKI-Discontinuation in CML Is Highly Predictive for Later Loss of MMR - a Report from after-SKI. Johan Richter (Lund)

16:08-16:20 Protein Transfer between CML and Bone Marrow Stromal Cells: Functional Meaning and the Involvement of Tnts Marta Kolba (Warsaw)

16:20-16:40 Discussion

16:40-16:50 Break

16:50-16:53 TESSA HOLYOAKE MEMORIAL LECTURE: SPECIAL LECTURE Chair: Steffen Koschmieder (Aachen)

16:53-16:56 Introduction

16:56-17:13 Understanding and targeting the stem cell origins of myeloid malignancies Ulrich Steidl (New York)

17:13-17:23 Discussion

17:23-17:26 Introduction

17:26-17:41 Keynote Presentation Regulation of hematopoietic stem and progenitor cells by the bone marrow microenvironment Ralf Adams (Munster)

17:41-17:51 Discussion

17:51-18:03 Selected Abstracts

17:51-18:03 The Role of the Autophagy Adaptor Protein PLEKHM1 in the Leukaemic Bone Marrow Microenvironment Christina Karantanou (Frankfurt)

18:03-18:15 Features of BCR-ABL1 Driven Myeloproliferative Disease in a CML Mouse Model Are Dependent on LSC Age and Fitness Hassan Almasoudi (Glasgow)

18:15-18:27 Murine Hematopoietic and Leukemic Stem/Progenitor Cell Lines As Model Systems for Bcr/Abl Driven Disease Veronika Sexl (Vienna)

18:27-18:39 Inhibition of PAK6-Mediated Survival and Cell Cycle Controls Selectively Targets Drug-Resistant CML Stem/Progenitor Cells Andrew Wu (Vancouver)


18:51-19:10 Discussion
19:10-19:50  JANET ROWLEY PRIZE CEREMONY

19:10-19:20  JANET ROWLEY PRIZE PRESENTATION
Presenter: Rob Welner (Birmingham)
Awardee 2020: Ravi Bhatia (Birmingham)

19:20-19:40  JANET ROWLEY PRIZE: SPECIAL LECTURE (2)
Chair: Rob Welner (Birmingham)
The seed and the soil – the leukemia stem cell in its niche and the quest for cure
Ravi Bhatia (Birmingham)

19:40-19:50  Discussion

Friday, October 2, 2020

11:30-12:15  SCIENTIFIC SESSION 3: IMMUNOLOGY AND INFLAMMATION
Chair: Shinya Kimura (Saga)

11:30-11:33  Introduction

Selected Abstracts

11:33-11:45  Sterile Inflammation in Normal Hematopoiesis and Leukemia
Pablo Llavona (Frankfurt)

11:45-11:57  Immunosuppressive Polarization of T Cell Subsets in Spleen and Bone Marrow of Mice with CML-like Disease
Julian Swatler (Warsaw)

11:57-12:15  Discussion

12:15-14:00 Lunch

13:00-14:00 SATELLITE SYMPOSIUM:
RECENT HIGHLIGHTS IN CML: WHAT HAVE WE LEARNED IN 2020?
Chair: Philippe Rousselot (France)
Speakers: Mario Tiribelli (Italy), Jorge Cortes (USA)

This educational symposium is organized and funded by Incyte Biosciences International Sàrl

14:00-15:33 SCIENTIFIC SESSION 4: THE BIOLOGY OF BLAST PHASE
Chair: Adam Mead (Oxford)

14:00-14:03 Introduction

14:03-14:18 Keynote Presentation
CRISPR predictions about relapse in CML
Daniel Bolon (Amherst)

14:18-14:28 Discussion

Selected Abstracts

14:28-14:40 RUNX1 Mutations in Blast Phase Chronic Myeloid Leukemia
Shady Adnan Awad (Helsinki)
14:40-14:52  H3K36ME3 Loss in Chronic Myeloid Leukemia Patients in Blast Crisis Is Mediated by an Unbalanced SETD2/KDM4A Activity and CAN be Therapeutically Targeted  Manuela Mancini (Bologna)

14:52-15:04  Releasing the Brakes on Apoptosis: Investigating Combinations of BH3 Mimetics and Nilotinib in Blast Phase CML  Narissa Parry (Glasgow)

15:04-15:16  Discovery of Novel CIP2A Variant (NOCIVA) and its Clinical Association with Myeloid Leukemias  Eleonora Mäkelä (Turku)

15:16-15:33  Discussion

15:35  Biology Posters - Mentored Poster Walk 1
Biology Poster Walk Leaders: Sin Tiong Ong (Singapore), Stéphane Prost (Fontenay-aux-Roses)

15:35 – 16:35  CONCURRENT MEET-THE-EXPERT SESSIONS (pre-registration required)
Clinical trial design  Donald Berry (Houston) and Joëlle Guilhot (Poitiers)
New drugs under development  Jorge Cortes (Augusta) and Tim Hughes (Adelaide)

16:35-16:45  Convene in the Main Conference Hall

16:45 -18:20  SPECIAL SYMPOSIUM ON ONCOIMMUNOLOGY IN HEMATOLOGIC MALIGNANCIES
Chairs: Jorge Cortes (Augusta), Kimmo Porkka (Helsinki)

16:45-16:55  Introductory overview – concepts and challenges

16:55-17:07  Leukemia vaccines  David Scheinberg (New York)

17:07-17:19  Past, present and future of CAR-T cell therapy for AML?  Saar Gill (Philadelphia)

17:19-17:31  Bi-specific and allogeneic CAR-T  Xianxin Hua (Philadelphia)

17:31-17:43  Checkpoint inhibitors  Justin Kline (Chicago)

17:43-17:55  Bispecific antibodies in myeloid leukemias  Roland Walter (Seattle)

17:55-18:20  General Discussion

18:20-18:53  SPECIAL LECTURE (3):
Chair: Rick Van Etten (Irvine)

18:20-18:23  Introduction

18:23-18:43  CML blast crisis and the microenvironment  Tannishtha Reya (San Diego)

18:43-18:53  Discussion
11:00-11:40 JOHN GOLDMAN PRIZE CEREMONY

11:00-11:10 JOHN GOLDMAN PRIZE PRESENTATION
Presenter: Hemant Malhotra (Jaipur)
Awardee 2020: Jane Apperley (London)

11:10-11:30 JOHN GOLDMAN PRIZE: SPECIAL LECTURE (4)
Chair: Hemant Malhotra (Jaipur)
CML and me: reflections on the roadmap to cure
Jane Apperley (London)

11:30-11:40 Discussion

11:40-13:10 SCIENTIFIC SESSION 5: NEW TREATMENT STRATEGIES
Chair: Gianantonio Rosti (Bologna), Tim Brümmendorf (Aachen)

11:40-11:43 Introduction

11:43-11:58 Keynote Presentation
Citius, Altius, Fortius ?
Andreas Hochhaus (Jena)

11:58-12:08 Discussion

Selected Abstracts

A Novel Oral Demethylating Agent, or-2100, in Combination with Tyrosine Kinase Inhibitors Overcomes Resistance in Chronic Myeloid Leukemia
Kazuharu Kamachi (Saga)
<table>
<thead>
<tr>
<th>Time</th>
<th>Title</th>
</tr>
</thead>
<tbody>
<tr>
<td>12:20-12:32</td>
<td>Targeting Integrated Stress Response Combined with Imatinib As a Novel Strategy to Eradicate Imatinib-Resistant CML Cells</td>
</tr>
<tr>
<td>12:32-12:44</td>
<td>Activity of Vodobatinib, a Novel Oral BCR-ABL1 Tyrosine Kinase Inhibitor (TKI) in CML Patients Failing TKI Therapies Including Ponatinib</td>
</tr>
<tr>
<td>12:44-12:56</td>
<td>Radotinib Treatment for Patients with Chronic Phase Chronic Myeloid Leukemia Resistant to or Intolerant of BCR-ABL1 Tyrosine-Kinase Inhibitors: 60-Month Follow-up of a Phase 2 Study</td>
</tr>
<tr>
<td>12:56-13:10</td>
<td>Discussion</td>
</tr>
<tr>
<td>13:10-14:50</td>
<td>Lunch</td>
</tr>
<tr>
<td>13:20-14:50</td>
<td>SATELLITE SYMPOSIUM: TRAVELING THROUGH THE UNSOLVED NEEDS IN CML: TOWARDS NEW STRATEGIES</td>
</tr>
<tr>
<td></td>
<td>Chair: Andreas Hochhaus (Jena)</td>
</tr>
<tr>
<td></td>
<td>Speakers: Valentin Garcia-Gutiérrez (Madrid), Massimo Breccia (Roma), Delphine Réa (Paris)</td>
</tr>
<tr>
<td></td>
<td><em>This educational symposium is organized and funded by Novartis Oncology</em></td>
</tr>
<tr>
<td>14:50-15:00</td>
<td>Convene in the Main Conference Hall</td>
</tr>
<tr>
<td>15:00-15:20</td>
<td>ESH INTERNATIONAL JOHN GOLDMAN RESEARCH AWARD CEREMONY</td>
</tr>
<tr>
<td>15:00-15:10</td>
<td>Presenter: Hervé Dombret (Paris), ESH President</td>
</tr>
<tr>
<td>15:10-15:20</td>
<td>Awardee 2020: Vaidehi Krishnan (Singapore)</td>
</tr>
<tr>
<td></td>
<td>High dimensional single-cell analysis to predict TFR at the time of CML diagnosis</td>
</tr>
<tr>
<td>15:20-16:55</td>
<td>SCIENTIFIC SESSION 6: TREATMENT-FREE REMISSION</td>
</tr>
<tr>
<td></td>
<td>Chair: Dragana Milojkovic (London)</td>
</tr>
<tr>
<td>15:23-15:38</td>
<td>Introduction</td>
</tr>
<tr>
<td>15:38-15:48</td>
<td>Discussion</td>
</tr>
<tr>
<td></td>
<td>Selected Abstracts</td>
</tr>
<tr>
<td>15:48-16:00</td>
<td>The rate of ( BCR-ABL1 ) decline after TKI commencement is a strong predictor of sustained Treatment Free Remission</td>
</tr>
<tr>
<td>16:00-16:12</td>
<td>High Level of Successful TKI Discontinuation Outside Clinical Trials - a Population-Based Study from the Swedish CML Registry</td>
</tr>
<tr>
<td>16:12-16:24</td>
<td>Efficacy and Safety of Pioglitazone in a Phase II Imatinib Discontinuation Trial (EDI-PIO) in Chronic Myeloid Leukemia Patients</td>
</tr>
<tr>
<td>Time</td>
<td>Session</td>
</tr>
<tr>
<td>--------------</td>
<td>-------------------------------------------------------------------------------------------</td>
</tr>
<tr>
<td>16:24-16:36</td>
<td>Update of the French Spirit Trial with a Detailed Focus on Treatment Free Remission</td>
</tr>
<tr>
<td>16:36-16:55</td>
<td>Discussion</td>
</tr>
<tr>
<td>16:55-17:05</td>
<td>Break</td>
</tr>
<tr>
<td>17:05-18:50</td>
<td><strong>SCIENTIFIC SESSION 7: TREATMENT-FREE REMISSION - PREDICTIVE FACTORS</strong></td>
</tr>
<tr>
<td></td>
<td>Chair: Delphine Réa (Paris)</td>
</tr>
<tr>
<td>17:05-17:08</td>
<td>Introduction</td>
</tr>
<tr>
<td>17:08-17:23</td>
<td><strong>Keynote Presentation</strong></td>
</tr>
<tr>
<td></td>
<td>Successful TFR in CML: is it predictable?</td>
</tr>
<tr>
<td>17:23-17:33</td>
<td>Discussion</td>
</tr>
<tr>
<td></td>
<td><strong>Selected Abstracts</strong></td>
</tr>
<tr>
<td>17:33-17:45</td>
<td>BCR-ABL1 Transcript Doubling Time after Imatinib Discontinuation for Treatment-Free Remission in Chronic Myeloid Leukemia in Chronic Phase: Early Predictive Surrogate for Treatment-Free Remission Failure after Imatinib Discontinuation</td>
</tr>
<tr>
<td>17:45-17:57</td>
<td>Chronic Myeloid Leukemia Patients’ Concerns during Phase I of the Treatment-Free Remission Journey: Considerations Between Patients and Doctors in the Different CML Advocates Network Regions</td>
</tr>
<tr>
<td>17:57-18:09</td>
<td>Best Duration of Deep Molecular Remission to Achieve Therapy-Free Remission in Chronic Myeloid Leukaemia</td>
</tr>
<tr>
<td>18:09-18:21</td>
<td>Risk Factor Analysis for Treatment-Free Remission Failure after Imatinib Discontinuation in Patients with Chronic Myeloid Leukemia in Chronic Phase: The Optimal Duration of Total Imatinib Treatment and MR4 Response</td>
</tr>
<tr>
<td>18:21-18:33</td>
<td>Discontinuation of Imatinib in Children with Chronic Myeloid Leukemia: An International Registry of Childhood Chronic Myeloid Leukemia (I-CML-Ped) Study</td>
</tr>
<tr>
<td>18:33-18:50</td>
<td>Discussion</td>
</tr>
<tr>
<td>18:50-19:23</td>
<td><strong>SPECIAL LECTURE (5):</strong></td>
</tr>
<tr>
<td></td>
<td>Chair: Giovanni Martinelli (Meldola)</td>
</tr>
<tr>
<td>18:50-18:53</td>
<td>Introduction</td>
</tr>
<tr>
<td>18:53-19:13</td>
<td>Human evolutionary genomic medicine</td>
</tr>
<tr>
<td>19:13-19:23</td>
<td>Discussion</td>
</tr>
</tbody>
</table>
14:30-16:30  WORKSHOP FOR NON-CLINICAL SCIENTISTS #2
ARTIFICIAL INTELLIGENCE / MACHINE LEARNING
Chairs: Michael Deininger (Salt Lake City) and Daniela Krause (Frankfurt)

14:30-14:35  Introduction
Michael Deininger (Salt Lake City) and Daniela Krause (Frankfurt)

14:35-15:00  Long-term single-cell quantification:
new tools for old questions
Timm Schroeder (Basel)

15:00-15:25  Computational Pathology: Towards Precision Medicine
Andrew Janowczyk (Cleveland)

15:25-15:50  Learning to Care: Artificial Intelligence
Meets Clinical Practice
Jeffrey Humpherys (Salt Lake City)

15:50-16:20  Discussion

16:20-16:30  Conclusion

14:30 – 15:30  CONCURRENT MEET-THE-EXPERT SESSIONS (pre-registration required)

Understanding and analyzing
the new ELN recommendations
Andreas Hochhaus (Jena) and Michael Mauro (New York)

15:30-16:30  Clinical Posters - Mentored Poster Walk 2
Clinical Poster Walk Leaders: Franck Nicolini (Lyon), Pierre Laneuville (Montreal)

16:30- 17:03  SPECIAL LECTURE (6):
Chair: Catriona Jamieson (La Jolla)

16:30-16:33  Introduction

16:33-16:53  Single cell RNA sequencing in MPN
Bethan Psaila (Oxford)

16:53-17:03  Discussion

17:03-18:20  DEBATE: “SWITCH TO A MORE POTENT DRUG TO GAIN ELIGIBILITY FOR TFR?”
Chair: Philippe Rousselot (Versailles)

17:03-17:06  Introduction

17:06-17:10  Voting

17:10-17:30  Against: Charles Schiffer (Detroit)

17:30-17:50  For: Giuseppe Saglio (Turin)

17:50-18:20  Voting and Discussion
18:20-19:00  
iCMLf PRIZE CEREMONY

18:20-18:30  
iCMLf PRIZE PRESENTATION:  
**Presenter:** Jerry Radich (Seattle)  
**Awardee 2020:** Sabira Kurtovic (Sarajevo)

18:30-18:50  
iCMLf PRIZE: SPECIAL LECTURE (7)  
**Chair:** Jerry Radich (Seattle)

CML management in war and peace: lessons that shaped future approaches  
Sabira Kurtovic (Sarajevo)

18:50-19:00  
Discussion

19:00-20:20  
**SCIENTIFIC SESSION 8: TKI SAFETY**  
**Chair:** Javier Pinilla-Ibarz (Tampa)

19:00-19:03  
Introduction

19:03-19:18  
**Keynote presentation**  
Novel trial designs  
Donald Berry (Houston)

19:18-19:28  
Discussion

**Selected Abstracts**

19:28-19:40  
Cellular Metabolic Flexibility to Explain Interindividual Susceptibility for Tyrosine Kinase Inhibitor-Induced Muscle Complaints  
Lando Janssen (Nijmegen)

19:40-19:52  
Retrospective Independent Review of Arterial Occlusive Events (AOEs) in the Ponatinib Phase 2 PACE Trial (NCT01207440) in Patients with Philadelphia Chromosome Positive (Ph+) Leukemia  
James Januzzi (Boston)

19:52-20:04  
High Body Mass Index (BMI) and Treatment Responses in Patients with Chronic Myeloid Leukemia in Chronic Phase (CML-CP) Receiving First-Line Dasatinib or Imatinib: Exploratory Post Hoc Analysis of the Phase 3 DASISION Study  
Massimo Breccia (Rome)

20:04-20:20  
Discussion

20:20  
Closing remarks