



**19th Annual John Goldman Conference on  
Chronic Myeloid Leukemia :  
Biology and Therapy**

**Estoril Congress Center, Estoril, Portugal  
October 12-15, 2017**

**Chairs : J. Cortes, T. Holyoake, T.P. Hughes**

**Co-Organizers : R. Bhatia, M. Copland, M. Deininger, D. Perrotti, J. Radich, D. Réa**

**Advisory Committee : J. Apperley, S. Branford, C. Gambacorti-Passerini, F. Guilhot, R. Hehlmann,  
P. Laneuville, F-X. Mahon, G. Saglio, C. Schiffer, S. Soverini, P. Valent, R. Van Etten**

**Thursday, October 12, 2017**

12:00-13:30	<i>Registration &amp; Poster Mounting</i>	
13:35-13:45	<b>WELCOME</b>	Jorge Cortes (Houston)
13:45-13:55	<b>iCMLf – A REVIEW</b>	Tim P. Hughes (Adelaide)
13:55-14:05	<b>JANET ROWLEY PRIZE PRESENTATION</b>	Connie Eaves (Vancouver)
	<b>Awardee 2017:</b> Tessa Holyoake (Glasgow)	
14:05-14:25	<b>SPECIAL LECTURE 1:</b> <i>(15' + 5' discussion)</i> <b>Chair:</b> Connie Eaves (Vancouver)	
	CML stem cells: from discovery to targeting	Tessa Holyoake (Glasgow)
14:25-15:10	<b>SCIENTIFIC SESSION 1: TOP SCORING ABSTRACTS 2017</b> <i>(12' + 3' discussion)</i> <b>Chair:</b> Richard A. Van Etten (Irvine)*, Anna M. Eiring (Salt Lake City)	
14:25-14:40	A second generation lysosomotropic agent and a NOVEL autophagy inhibitor drive cml stem cell differentiation and sensitize them to TKI treatment <i>in vitro and in vivo</i>	Pablo Baquero (Glasgow)
14:40-14:55	Role of SCF-expressing bone marrow populations in hematopoietic and leukemic stem cell regulation	Mansi Shah (Birmingham)
14:55-15:10	Incidence of secondary malignancies in patients with chronic myeloid leukemia in chronic phase (CML-CP) in the era of tyrosine kinase inhibitors (TKI)	Koji Sasaki (Houston)
15:10-15:40	<i>Coffee Break</i>	

15:40-17:15

## SCIENTIFIC SESSION 2: STEM CELLS AND THE MICROENVIRONMENT

Chair: Xiaoyan Jiang (Vancouver)

15:40-16:00

### **Keynote Presentation (15' + 5' discussion)**

Therapy resistant CML stem cells are dependent on mitochondrial oxidative metabolism for their survival

G. Vignir Helgason (Glasgow)

16:00-17:15

### **Selected Abstracts (12' + 3' discussion)**

16:00-16:15

Single-cell molecular analysis defines therapy response and immunophenotype of stem cell subpopulations in CML

Rebecca Warfvinge (Lund)

16:15-16:30

Control of leukaemic stem cells in the vascular niche in chronic myeloid leukaemia by e-selectin, CD44, SCL/Tal1 and BCR-ABL1

Daniela S. Krause (Frankfurt)

16:30-16:45

KLF4-mediated suppression of DYRK2 enables self-renewal and survival of leukemia stem cells in chronic myeloid leukemia

Daniel Lacorazza (Houston)

16:45-17:00

CXCL12-expressing bone marrow niches differentially regulate CML stem cells

Puneet Agarwal (Birmingham)

17:00-17:15

Predicting response to TKI therapy using single cell expression data from leukaemic stem cells

Lisa E.M. Hopcroft (Glasgow)

17:15

*Welcome reception and poster viewing*

**Friday, October 13, 2017**

8:00-10:00

## WORKSHOP FOR NON-CLINICAL SCIENTISTS #1 METABOLOMICS

Chairs: Aaron Schimmer (Toronto) and Mike Deininger (Salt Lake City)

8:00-8:05

Introduction

8:05-8:30

Novel mitochondrial and metabolic pathways that regulate the fate and differentiation of leukemia

Aaron Schimmer (Toronto)

8:30-8:40

General Discussion

8:40-9:05

Signaling and targeting of leukemia metabolism

Jing Chen (Atlanta)

9:05-9:15

General Discussion

9:15-9:40

Reprogrammed amino acid metabolism in CML progression

Takahiro Ito (Athens, USA)

9:40-9:50

General Discussion

9:50-10:00

Concluding Remarks

10:00-10:20

*Coffee Break*

10:20-10:30

## SESSION LEUKA-ESH: LEUKA INTERNATIONAL SCHOLARSHIP AWARD CEREMONY

10:30-11:50

## SCIENTIFIC SESSION 3: MOLECULAR BIOLOGY AND NEW TARGETS

Chair: Steffen Koschmieder (Aachen)

10:30-10:50

### **Keynote Presentation (15' + 5' discussion)**

The BM niche uses Mir-300 as a biological rheostat to selectively control stem cell-driven malignant hematopoiesis and innate anti-cancer immunity

Danilo Perrotti (Baltimore)

<b>10:50-11:50</b>	<b><i>Selected Abstracts (12' + 3' discussion)</i></b>	
10:50-11:05	Nuclear Vav3 upregulation is required for BCR-ABL lymphoid leukemogenesis through polycomb repression complex dependent repression	Ramesh Nayak (Cincinnati)*
11:05-11:20	MYC drives epigenetic control of MIR150 gene regulation in CML	Pavel Burda (Prague)
11:20-11:35	CIP2A regulates PP2A by altering the profile of PP2A B subunits	Claire Lucas (Liverpool)
11:35-11:50	Structural and functional analysis of the Bcr-Abl DH and Ph domains: closing the gaps towards a comprehensive molecular understanding of p210 and p190 Bcr-Abl signaling?	Oliver Hantschel (Lausanne)
<b>11:50-12:10</b>	<b>SPECIAL LECTURE 2: (15' + 5' discussion)</b> <b>Chair: TBA</b>	
	Metabolic regulation of blood and bone forming stem cells: The role of ROS and nitric oxide	Tsvee Lapidot (Rehovot)
<b>12:10-14:10</b>	<b><i>Lunch and Poster Viewing</i></b>	
<b>14:10-15:15</b>	<b>SCIENTIFIC SESSION 4: CML-BP AND MECHANISMS OF TRANSFORMATION</b> <b>Chair: Simona Soverini (Bologna)</b>	
<b>14:10-14:30</b>	<b><i>Keynote Presentation (15' + 5' discussion)</i></b> Blast phase and residual CML. More in common that we think?	Michael Deininger (Salt Lake City)
<b>14:30-15:15</b>	<b><i>Selected Abstracts (12' + 3' discussion)</i></b>	
14:30-14:45	The signaling axis atypical protein kinase C $\lambda/\iota$ -Satb2 mediates blastic phase transformation of b-cell precursors	Ramesh Nayak (Cincinnati)*
14:45-15:00	Role of somatic mutations in clonal evolution of CML from chronic phase to blast phase	Chetasi Talati (Tampa)*
15:00-15:15	Loss of G0S2 contributes to kinase-independent TKI resistance and blastic transformation in CML	Anna M. Eiring (Salt Lake City)
<b>15:15-15:45</b>	<b><i>Coffee Break</i></b>	
<b>15:45-16:05</b>	<b>SPECIAL LECTURE 3: (15' + 5' discussion)</b> <b>Chair: Stephane Prost (Fontenay-Aux-Roses)</b>	
	Characterization of ALL Stem Cells	Irmela Jeremias (Munich)
<b>16:05-17:10</b>	<b>SCIENTIFIC SESSION 5: MECHANISMS OF RESISTANCE AND NEW TARGETS</b> <b>Chair: Giuseppe Saglio (Turin)</b>	
<b>16:05-16:25</b>	<b><i>Keynote Presentation (15' + 5' discussion)</i></b> Outflanking resistance with ABL001	Andrew Wylie

<b>16:25-17:10</b>	<b><i>Selected Abstracts (12' + 3' discussion)</i></b>	
16:25-16:40	In bone marrow of CML TKI-resistant patients BMP pathway alterations persist and LSC implement an autocrine loop that favor their treatment escape	Véronique Maguer Satta (Lyon)
16:40-16:55	Targeting autophagy in chronic myeloid leukemia through Inhibition of the core autophagy protein ATG4B	Vanessa L. Porter (Vancouver)
16:55-17:10	Activation of p53 enhances antileukemia activity of Bcr-Abl tyrosine kinase inhibitors in a murine model of CML	Bing Z. Carter (Houston)
<b>17:10</b>	<b><i>Poster Walk 1 and Refreshments</i></b>	
	<b>Biology Poster Walk:</b> Catriona Jamieson (La Jolla) and Oliver Hantschel (Lausanne)	
	<b>Clinical Poster Walk :</b> Franck-Emmanuel Nicolini (Lyon) and Michael Mauro (New York)	

## Saturday, October 14, 2017

<b>9:00-11:00</b>	<b>WORKSHOP FOR NON-CLINICAL SCIENTISTS #2</b>	
	<b>CELL FATE TRACKING</b>	
	<b>Chairs:</b> Ravi Bhatia (Birmingham) and Daniela Krause (Frankfurt)	
9:00-9:05	Introduction	
9:05-9:30	Fate mapping of hematopoietic stem cell activity in situ	Ann-Kathrin Schuon (Heidelberg)
9:30-9:40	General Discussion	
9:40-10:05	Quantitative single-cell imaging technologies to study hematopoietic stem cells and their microenvironment	Konstantinos Kokkaliaris (Basel)
10:05-10:15	General Discussion	
10:15-10:40	In vivo tools to follow the fate and function of hematopoietic stem cells	David Traver (San Diego)
10:40-10:50	General Discussion	
10:50-11:00	Concluding Remarks	

8:30-11:10

**SYMPOSIUM ON MYELOPROLIFERATIVE NEOPLASMS**

**Chairs:** Carlo Gambacorti-Passerini (Monza)\* and Amy Klion (Bethesda)\*

8:30-8:40

**Welcome and Introduction**

8:40-8:55

Current understanding of the biology of MPN

Jyoti Nangalia (Cambridge)

8:55-9:05

Discussion

9:05-9:20

Understanding eosinophilic syndromes and their management

Amy Klion (Bethesda)

9:20-9:30

Discussion

9:30-9:45

Mast cell diseases : Biology and management

Celal Ustun (Minneapolis)

9:45-9:55

Discussion

9:55-10:10

MDS/MPN : a real entity?

Eric Padron (Tampa)

10:10-10:20

Discussion

10:20-10:35

Starting to understand CNL/aCML as a new entity

Carlo Gambacorti-Passerini

10:35-10:45

Discussion

(Monza)

10:45-11:00

JMML : not just a subset of MDS anymore.

Charlotte Niemeyer (Freiburg)

11:00-11:10

Discussion

11:00-11:30

**Coffee Break**

11:30-12:12

**BRIEF ORAL COMMUNICATIONS (BIOLOGY) (4 slides/5' + 2' discussion)**

**Chair:** Mhairi Copland (Glasgow)

11:30-11:37

Icsbp-mediated deficiency of Nore1A contributes to CML leukemogenesis by promoting apoptosis resistance

Olatundun Williams (Chicago)

11:37-11:44

Identification of progression-associated mutations in CML by comparative genomic analysis

Shady Adnan-Awad (Helsinki)

11:44-11:51

High BCR-ABL expression defines CD34+ progenitors with significant differences in signal transduction, short- and long-term proliferative potential

Paolo Vigneri (Catania)

11:51-11:58

Effects of ponatinib on vascular endothelial cells as possible explanation for drug-related adverse events seen in patients with CML

Emir Hadzijušević (Vienna)

11:58-12:05

Genomic characterization of CML at diagnosis reveals preexisting somatic mutations that may predict progression to blastic phase independently of BCR-ABL1 mutations

Marcin Machnicki (Warsaw)

12:05-12:12

Expression profiles of transcribed ultraconserved noncoding RNAs (T-UCRs) associated with molecular response in CML

Paulo Rodrigues-Santos (Coimbra)

<b>12:12-12:30</b>	<b>BRIEF ORAL COMMUNICATIONS (CLINICAL) (4 slides/5' + 2' discussion)</b> <b>Chair:</b> Tim Brummendorf (Aachen) and Jane Apperley (London)*	
12:12-12:19	The next-in-CML study: creating a national network of laboratories performing BCR-ABL1 kinase domain mutation screening by next generation amplicon deep sequencing	Simona Soverini (Bologna)
12:19-12:26	Bosutinib vs imatinib for newly diagnosed chronic myeloid leukemia in the BFORE trial: results by 3-month BCR-ABL1 transcript level	Andreas Hochhaus (Jena)
<b>12:30-14:30</b>	<b>Lunch and Poster Viewing</b>	
<b>14:30-15:35</b>	<b>SCIENTIFIC SESSION 6: BIOMARKERS</b> <b>Chair:</b> Nick Cross (Southampton)	
<b>14:30-14:50</b>	<b>Keynote Presentation (15' + 5' discussion)</b> Integrative genomics at diagnosis of CML reveals a higher incidence of cancer associated mutations in patients with poor outcome to tyrosine kinase inhibitor therapy in a study of patients with an optimal response versus a very poor response	Susan Branford (Adelaide)
<b>14:50-15:35</b>	<b>Selected Abstracts (12' + 3' discussion)</b>	
14:50-15:05	Somatic variants in epigenetic modifiers identified at diagnosis can predict high risk CML-CP patients starting treatment with first but not second generation TKI	Georgios Nteliopoulos (London)*
15:05-15:20	Allelic polymorphisms of KIR and HLA predict favorable responses to tyrosine kinase inhibitor in CML	Shinya Kimura (Saga)
15:20-15:35	Flow-cytometry peripheral blood CD26+ leukemia stem cells quantification in CML patients during TKI therapy	Monica Bocchia (Siena)
<b>15:35-16:05</b>	<b>Coffee Break</b>	
<b>16:05-16:25</b>	<b>SPECIAL LECTURE 4: (15' + 5' discussion)</b> <b>Chair:</b> TBA	
	Chimeric antigen receptor t-cell therapy for hematologic malignancies	James Kochenderfer (Bethesda)
<b>16:25-17:45</b>	<b>SCIENTIFIC SESSION 7: THERAPEUTIC INTERVENTIONS</b> <b>Chair:</b> Michael Mauro (New York)	
<b>16:25-16:45</b>	<b>Keynote Presentation (15' + 5' discussion)</b> The new ELN recommendations	Andreas Hochhaus (Jena)
<b>16:45-17:30</b>	<b>Selected Abstracts (12' + 3' discussion)</b>	
16:45-17:00	Assessment of imatinib as first-line treatment of CML: 10-Year survival results of the randomized CML study IV and impact of non-CML determinants	Rüdiger Hehlmann (Mannheim)
17:00-17:15	Clinical impact of early and deeper molecular response in newly diagnosed chronic phase CML patients treated with radotinib 300mg BID or imatinib-2 year follow up data of rerise study	Young Rok Do (Daegu)

17:15-17:30	Bosutinib vs imatinib for newly diagnosed CML in the BFORE trial: 18 month follow-up	Carlo Gambacorti-Passerini (Monza)*
17:30-17:45	Response to front-line imatinib treatment in children and adolescents with CML – data from a large pediatric cohort	Meinolf Suttorp (Dresden)
<b>17:45-17:55</b>	<b>JOHN GOLDMAN PRIZE PRESENTATION</b> <b>Awardee 2017:</b> Tim P. Hughes (Adelaide)	Jorge Cortes (Houston)
<b>17:55-18:15</b>	<b>SPECIAL LECTURE 5 (15' + 5' discussion)</b> <b>Chair:</b> TBA  From Safe haven to functional cure	Tim P. Hughes (Adelaide)

## Sunday, October 15, 2017

**7:00-8:00** **FUN RUN - PRE-REGISTRATION IS REQUIRED**  
*In memory of Jean Khoury*

<b>8:30-10:00</b>	<b>WORKSHOP FOR NON-CLINICAL SCIENTISTS #3</b> <b>BIOINFORMATICS AND MODELING – PERSPECTIVES FOR SYSTEMS MEDICINE IN CML</b> <b>Chair:</b> Ingo Roeder (Dresden)	
8:30-8:35	Introduction	
8:35-8:55	Single cell'omics analyses: why and how?	Lisa E. M. Hopcroft (Glasgow)
8:55-9:02	General Discussion	
9:02-9:22	Prospects for linking TKI protein kinase inhibition profiles to CML modelling parameters	Richard A. Engh (Tromsø)
9:22-9:29	General Discussion	
9:29-9:49	From maths to bedside: Model-based treatment optimization	Ingo Roeder (Dresden)
9:49-9:56	General Discussion	
9:56-10:00	Concluding Remarks	
<b>10:00-11:05</b>	<b>SCIENTIFIC SESSION 8: CLINICAL TRIALS INCLUDING TREATMENT FREE REMISSION</b> <b>Chair:</b> Delphine Rea (Paris) and Richard Clark (Liverpool)	
<b>10:00-10:20</b>	<b>Keynote Presentation (15' + 5' discussion)</b> The promises and challenges of TFR	Robert Peter Gale (London)
<b>10:20-11:35</b>	<b>Selected Abstracts (12' + 3' discussion)</b>	
10:20-10:35	Interim analysis of the canadian TKI discontinuation trial: treatment free remission accomplished by dasatinib (TRAD)	Dennis Dong Hwan Kim (Toronto)
10:35-10:50	Interim results from the U.S. Life after stopping TKIs (LAST) study	Ehab Atallah (Milwaukee)
10:50-11:05	Efficacy, safety, and landmark analysis in heavily pretreated patients with chronic-phase CML: 5-year updates from the pivotal phase 2 ponatinib PACE trial	Jorge Cortes (Houston)

<b>11:05-12:10</b>	<b>SCIENTIFIC SESSION 9: ADHERENCE AND PREVALENCE</b> <b>Chair:</b> Franck-Emmanuel Nicolini (Lyon)	
<b>11:05-11:25</b>	<b>Keynote Presentation</b> (15' + 5' discussion)	<b>TBA</b>
<b>11:25-12:10</b>	<b>Selected Abstracts</b> (12' + 3' discussion)	
11:25-11:40	Factors influencing adherence in CML and ways to improvement: Results of a patient-driven survey of 2546 patients in 63 countries	Giora Sharf (Netanya)
11:40-11:55	The Potential health gain and cost savings of improving adherence in chronic myeloid leukemia	Tim Govers (Nijmegen)
11:55-12:10	Burden of CML in China and India: the rising incidence and prevalence of the disease	Marc Delord (Paris)
<b>12:10-12:20</b>	<b>iCMLf PRIZE (Emerging regions support &amp; partnership) PRESENTATION:</b> <b>Awardee 2017:</b> Jerry Radich (Seattle)	<b>TBA</b>
<b>12:20-12:40</b>	<b>SPECIAL LECTURE 6:</b> (15' + 5' discussion) <b>Chair:</b> TBA	
	WEIRD solutions to real world problems	Jerry Radich (Seattle)
<b>12:40</b>	<b>Meeting Adjourned</b>	