

**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**

<b>12:00-13:30</b>	<i>Registration &amp; Poster Mounting</i>	
<b>13:35-13:45</b>	<b>WELCOME</b>	Jorge Cortes (Houston)
<b>13:45-13:55</b>	<b>iCMLf – A REVIEW</b>	Tim P. Hughes (Adelaide)
<b>13:55-14:05</b>	<b>JANET ROWLEY PRIZE PRESENTATION</b> Awardee 2017: Tessa Holyoake (Glasgow)	
<b>14:05-14:25</b>	<b>SPECIAL LECTURE 1: (15' + 5' discussion)</b> Chair: Connie Eaves (Vancouver)	
	CML stem cells: from discovery to targeting	Tessa Holyoake (Glasgow)
<b>14:25-15:10</b>	<b>SCIENTIFIC SESSION 1: TOP SCORING ABSTRACTS 2017 (12' + 3' discussion)</b> Chairs: 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, USA)
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)
<b>15:10-15:40</b>	<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 leukemic stem cells in the vascular niche in chronic myeloid leukemia 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, USA)

17:00-17:15

Predicting response to TKI therapy using single cell expression data from leukemic 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*

8:00-10:00

**SATELLITE SYMPOSIUM**

**HOT TOPICS FOR CML IN 2017: A PATIENT JOURNEY**

**Chairs:** Jeroen Janssen (Amsterdam) and Franck-Emmanuel Nicolini (Lyon)

**Speakers:** Fausto Castagnetti (Bologna), Jeroen Janssen (Amsterdam), Martin Müller (Mannheim), Franck-Emmanuel Nicolini (Lyon), Susanne Saussele (Heidelberg)

*This educational symposium is organized and funded by Incyte Biosciences*

10:20-10:30

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

**Chair:** Nick Cross (Southampton)

**Awardees 2017:** Lorna Jackson (Glasgow), Mansi Manij Shah (Birmingham, USA), Marcin M. Machnicki (Warsaw), Vanessa L. Porter (Vancouver), Chetasi Talati (Tampa), Flavia Vasconcelos (Rio de Janeiro), Angela McLigeyo (Nairobi), Aditya Singh (Punjab), Tanmoy Kumar Mandal (Mumbai), Co Nguyen Phuong Dung (Ho Chi Minh City)

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)

10:50-11:50

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

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)

11:50-12:10

**SPECIAL LECTURE 2: (15' + 5' discussion)**

**Chair:** Michele Bacarani (Bologna)

Metabolic regulation of blood and bone forming stem cells: The role of ROS and nitric oxide

Tsvee Lapidot (Rehovot)

12:10-14:10

**Lunch and Poster Viewing**

12:40-14:10

**MID-DAY SATELLITE SYMPOSIUM**

**THE NEXT FRONTIER IN CHRONIC MYELOID LEUKEMIA RESEARCH - THE PATIENT'S PATH**

**Chairs:** Dominik Wolf (Bonn), Andreas Burchert (Marburg)

**Speakers:** Andreas Burchert (Marburg), Lia Gore (Aurora), Dominik Wolf (Bonn)

*This educational symposium is organized and funded by Bristol-Myers Squibb*

14:10-15:15

**SCIENTIFIC SESSION 4: CML-BP AND MECHANISMS OF TRANSFORMATION**

**Chair:** Simona Soverini (Bologna)

14:10-14:30

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

Blast phase and residual CML. More in common than we think?

Michael Deininger (Salt Lake City)

14:30-15:15

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

14:30-14:45

The signaling axis atypical protein kinase C  $\lambda$ /I-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 GOS2 contributes to kinase-independent TKI resistance and blastic transformation in CML

Anna M. Eiring (Salt Lake City)

15:15-15:45

**Coffee Break**

15:45-16:05

**SPECIAL LECTURE 3: (15' + 5' discussion)**

**Chair:** Stéphane Prost (Fontenay-Aux-Roses)

Characterization of ALL Stem Cells

Irmela Jeremias (Munich)

16:05-17:10

**SCIENTIFIC SESSION 5: MECHANISMS OF RESISTANCE AND NEW TARGETS**

**Chair:** Giuseppe Saglio (Turin)

16:05-16:25

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

Outflanking resistance with ABL001

Andrew Wylie (Cambridge)

16:25-17:10

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

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)

17:10

**Poster Walk 1 and Refreshments**

**Biology Poster Walk Leaders:** Catriona Jamieson (La Jolla) and Oliver Hantschel (Lausanne)

**Clinical Poster Walk Leaders :** Franck-Emmanuel Nicolini (Lyon) and Michael Mauro (New York)

**Saturday, October 14, 2017**

**8:00-10:50**

**SYMPOSIUM ON MYELOPROLIFERATIVE NEOPLASMS**

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

**8:00-8:10**

**Welcome and Introduction**

8:10-8:25

Current understanding of the biology of MPN

Jyoti Nangalia (Cambridge)

8:25-8:35

Discussion

8:35-8:50

MDS/MPN: a real entity?

Eric Padron (Tampa)

8:50-9:00

Discussion

9:00-9:15

Understanding eosinophilic syndromes and their management

Amy Klion (Bethesda)

9:15-9:25

Discussion

9:25-9:40

Mast cell diseases: biology and management

Celal Ustun (Minneapolis)

9:40-9:50

Discussion

9:50-10:05

Starting to understand CNL/aCML as a new entity

Carlo Gambacorti-Passerini (Monza)

10:05-10:15

Discussion

10:15-10:30

JMML: not just a subset of MDS anymore

Ayami Yoshimi-Nöllke (Freiburg)

10:30-10:40

Discussion

10:40-10:50

General Discussion

**9:00-11:00**

**WORKSHOP FOR NON-CLINICAL SCIENTISTS #2**

**CELL FATE TRACKING**

**Chairs:** Ravi Bhatia (Birmingham, USA) 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

9:30-9:40

General Discussion

(Heidelberg)

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

**10:50-11:30**

*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 Hadzijusufovic (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>Chairs:</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 CML in the BFORE trial: results by 3-month BCR-ABL1 transcript level	Andreas Hochhaus (Jena)

**12:30-14:30** *Lunch and Poster Viewing*

**13:00-14:30** **MID-DAY SATELLITE SYMPOSIUM**  
**AIMING FOR LONG-TERM TREATMENT EFFECTIVENESS:  
INDIVIDUALISING THERAPY IN CML**  
**Chair:** Jorge Cortes (Houston)  
**Speakers:** Massimo Breccia (Rome), Rita O. Christensen (Denmark), Jorge Cortes (Houston)

*This educational symposium is organized and funded by Pfizer Oncology*

**14:30-15:35** **SCIENTIFIC SESSION 6: BIOMARKERS**  
**Chair:** Nick Cross (Southampton)

**14:30-14:50** ***Keynote Presentation (15' + 5' discussion)***  
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)

**14:50-15:35** ***Selected Abstracts (12' + 3' discussion)***

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><i>Coffee Break</i></b>	
<b>16:05-16:25</b>	<b>SPECIAL LECTURE 4: (15' + 5' discussion)</b> <b>Chair:</b> Philippe Rousselot (Versailles)	
	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>16:45-17:45</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 Suttrop (Dresden)
<b>17:45-17:55</b>	<b>JOHN GOLDMAN PRIZE PRESENTATION</b> <b>Awardee 2017:</b> Tim P. Hughes (Adelaide)	
<b>17:55-18:15</b>	<b>SPECIAL LECTURE 5: (15' + 5' discussion)</b> <b>Chair:</b> Jorge Cortes (Houston)	
	From Safe haven to functional cure	Tim P. Hughes (Adelaide)

**18:15-19:45**

**SATELLITE SYMPOSIUM**

**TREATMENT-FREE REMISSION IN CML: IS IT TIME TO TAKE THE LEAP?**

**Chair:** Tim P. Hughes (Adelaide)

**Speakers:** Tim P. Hughes, Delphine Réa (Paris), Giuseppe Saglio (Turin)

*This educational symposium is organized by prIME Oncology with the support of Novartis Oncology*

**Sunday, October 15, 2017**

**7:00-8:00**

**FUN RUN - PRE-REGISTRATION IS REQUIRED**

*In memory of Jean Khoury*

**8:30-10:00**

**SATELLITE SYMPOSIUM**

**OPTIMIZING TREATMENT OF PATIENTS WITH CHRONIC MYELOPROLIFERATIVE NEOPLASMS: GOALS, MILESTONES & MORE**

**Chairs:** Tariq Mughal (Boston), Giuseppe Saglio (Turin)

**Speakers:** Jorge Cortes (Houston), Michael Deininger (Salt Lake City), Steffen Koschmieder (Aachen), François-Xavier Mahon (Bordeaux), Jerry Radich (Seattle)

*This educational symposium is organized and funded by Alpine Oncology Foundation*

**8:30-10:00**

**WORKSHOP FOR NON-CLINICAL SCIENTISTS #3**

**BIOINFORMATICS AND MODELING – PERSPECTIVES FOR SYSTEMS MEDICINE IN CML**

**Chair:** 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 (Tromsøe)

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

**10:00-11:05**

**SCIENTIFIC SESSION 8: CLINICAL TRIALS INCLUDING TREATMENT FREE REMISSION**

**Chairs:** Delphine Réa (Paris) and Richard Clark (Liverpool)

**10:00-10:20**

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

The promises and challenges of TFR

Robert Peter Gale (London)

**10:20-11:35**

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

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 (15' + 5' discussion)</b> Incorporating PRO in patient management: does it help?	Laura Cannella (Rome)
<b>11:25-12:10</b>	<b>Selected Abstracts (12' + 3' discussion)</b>	
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	Jana Pelouchova (Prague)
11:40-11:55	The potential health gain and cost savings of improving adherence in CML	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>12:20-12:40</b>	<b>SPECIAL LECTURE 6: (15' + 5' discussion)</b> <b>Chair:</b> Pat Garcia-Gonzalez (Seattle)	
	WEIRD solutions to real world problems	Jerry Radich (Seattle)
<b>12:40</b>	<b>Meeting Adjourned</b>	