

## 18th Annual John Goldman Conference on Chronic Myeloid Leukemia: Biology and Therapy

**Royal Sonesta Hotel – Houston, Texas, USA**  
**September 15-18, 2016**

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

**Co-Organizers:** R. Bhatia, M. Copland, M. Deininger, P. Hari, 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

*[Scientific Sessions will be held in the Legends Ballroom, unless otherwise noted]*

### Thursday, September 15, 2016

10:00 AM-12:30 PM	Poster Mounting <i>[Legends Ballroom Foyer]</i>	
11:00 AM-12:30 PM	Registration <i>[Legends Ballroom Foyer]</i>	
12:30 PM-12:40 PM	<b>WELCOME TO HOUSTON</b> <i>[Legends Ballroom]</i>	Jorge Cortes (Houston)
	Special Guest : Ronald DePinho, MD, President, MD Anderson Cancer Center	
12:40 PM-12:50 PM	<b>iCMLf – A REVIEW</b>	Tim P. Hughes (Adelaide)
12:50 PM-1:00 PM	<b>JANET ROWLEY PRIZE PRESENTATION</b>	Ravi Bhatia (Birmingham)
	Awardees 2016: Nora Heisterkamp (Los Angeles), John Groffen (Los Angeles)	
1:00 PM-1:20 PM	<b><u>SPECIAL LECTURE 1:</u></b> <i>(15' + 5' discussion)</i>	
	Chair: Ravi Bhatia (Birmingham)	
	Evaluation in leukemia research: from identification of the BCR/ABL fusion to carbohydrate-mediated protection by the bone marrow microenvironment	Nora Heisterkamp (Los Angeles)
1:20 PM-2:50 PM	<b><u>SCIENTIFIC SESSION 1: TOP SCORING ABSTRACTS 2016</u></b> <i>(12' + 3' discussion)</i>	
	Chairs: Danilo Perrotti (Baltimore), Stéphane Prost (Fontenay-aux-Roses)	
1:20-1:35	MS4A3: a new player in CML stem cell survival and myeloid malignancies	Anna M. Eiring (Salt Lake City)
1:35-1:50	Comprehensive analysis of the structural, biochemical and signaling differences of the p210 and p185 isoforms of Bcr-Abl in CML and B-ALL	Oliver Hantschel (Lausanne)
1:50-2:05	CML stem cells are dependent on mitochondrial oxidative metabolism for their survival	G. Vignir Helgason (Glasgow)
2:05-2:20	A potential role for interactions with the bone marrow microenvironment in regulating the aggressivity of BCR-ABL1 – T315I-positive chronic myelogenous leukemia	Daniela S. Krause (Frankfurt)

2:20-2:35	Natural killer cells and molecular relapse upon imatinib discontinuation in patients with chronic myeloid leukemia: results from the French multicenter Immunostim study	Delphine Réa (Paris)
2:35-2:50	Prediction for sustained deep molecular response of BCR-ABL levels in patients with chronic myeloid leukemia in chronic phase	Koji Sasaki (Houston)
<b>2:50 PM-3:20 PM</b>	<b><i>Coffee Break [Legends Ballroom Foyer]</i></b>	
<b>3:20 PM-5:10 PM</b>	<b><u>SCIENTIFIC SESSION 2: STEM CELLS AND THE MICROENVIRONMENT</u></b> <b>Chair:</b> Mirle Schemionek (Aachen)	
<b>3:20-3:40</b>	<b><u>Keynote Presentation</u> (15' + 5' discussion)</b> Partners in crime: dissecting CML stem cells and microenvironmental interactions	Ravi Bhatia (Birmingham)
	<b><u>Selected Abstracts</u> (12' + 3' discussion)</b>	
3:40-3:55	Leukomics: Bringing high-throughput stem cell data to the leukemia research community	Lorna Jackson (Glasgow)
3:55-4:10	Combined inhibition of beta-catenin signaling and Bcr-Abl tyrosine kinase synergistically targets blast crisis CML cells and stem/progenitor cells in vitro and in vivo	Bing Z. Carter (Houston)
4:10-4:25	Potential targeting Ph+ acute lymphoblastic leukemia stem and progenitor cells by modulating the CIP2A-SET-SETBP1-mediated suppression of PP2A activity	Danilo Perrotti (Baltimore)
4:25-4:40	Phosphorylation of eIF2alpha as novel mechanism regulating secretion of cytokines and matrix-modifying enzymes by CML cells to support the microenvironment changes and leukemia-stroma interactions	Katarzyna Piwocka (Warsaw)
4:40-4:55	The BMP pathway deregulations in the CML niche persist under tyrosine kinase inhibitors and induce LSC persistence	Elodie Grockowiak (Lyon)
4:55-5:10	CML-induced dysregulation of bone marrow skeletal stem cell subpopulations	Puneet Agarwal (Birmingham)

**5:10 PM-5:40 PM** ***Afternoon Refreshment Break [Discovery Ballroom Foyer]***

**5:40 PM-6:40 PM**

**PRODUCT THEATER [Founders Ballroom III-IV]**

Presented by Novartis

*Attendees may wish to attend non-CME accredited seminars, as advertised by sponsors and posted near the Conference registration area.*

**5:40 PM-7:10 PM**

***Welcome Reception and Poster Viewing [Discovery Ballroom & Foyer]***

## Friday, September 16, 2016

**7:00 AM-8:15 AM** *Continental Breakfast and Poster Viewing [Discovery Ballroom & Foyer]*

**8:00 AM-10:00 AM** **WORKSHOP FOR NON-CLINICAL SCIENTISTS #1** *[Founders Ballroom I-II]*

### **SINGLE CELL APPROACHES**

**Chair:** Jerry Radich (Seattle)

8:00-8:05	Introduction	Jerry Radich (Seattle)
8:05-8:30	Drop-seq analysis of early fate decisions in human hematopoiesis	Rahul Satija (New York)
8:30-8:40	Discussion	
8:40-9:05	Heterogeneity and population dynamics of the chronic myeloid leukemia microenvironment	Robert Welner (Birmingham)
9:05-9:15	Discussion	
9:15-9:40	Single-cell transcriptomics uncovers heterogeneity and distinct molecular signatures of therapy-resistant chronic myeloid leukemia stem cells	Adam Mead (Oxford)
9:40-9:50	Discussion	
9:50-10:00	Concluding remarks	Jerry Radich (Seattle)

**8:30 AM-9:30 AM**

### **PRODUCT THEATER** *[Founders Ballroom III-IV]*

Presented by Cepheid

*Attendees may wish to attend non-CME accredited seminars, as advertised by sponsors and posted near the Conference registration area.*

**10:00 AM-10:30 AM** *Coffee Break [Legends Ballroom Foyer]*

**10:30 AM-11:50 AM** **SCIENTIFIC SESSION 3: MOLECULAR BIOLOGY AND NEW TARGETS (1)**

**Chair:** Giovanni Martinelli (Bologna)

<b>10:30-10:50</b>	<b><u>Keynote Presentation</u></b> <i>(15' + 5' discussion)</i> ACTIW : a program to validate candidate molecules able to target minimal residual disease in CML	Philippe Rousselot (Versailles)
	<b><u>Selected Abstracts</u></b> <i>(12' + 3' discussion)</i>	
10:50-11:05	High or low BCR-ABL transcripts at diagnosis are associated with different phosphorylation of downstream targets and distinct short- and long-term proliferative potential	Paolo Vigneri (Catania)
11:05-11:20	Identification of imatinib-sensitizing genes in chronic myeloid leukemia with a genome-scale Crispr knock-out screen	Matthieu Lewis (Bordeaux)
11:20-11:35	Regulative loop between beta-catenin and protein tyrosine phosphatase receptor type gamma (PTPRG) in chronic myeloid leukemia	Luisa Tomasello (Verona)

11:35-11:50	Hypoxia inducible arginase ARG2 is required for the survival of acute and chronic myeloid leukemia cells	King Pan Ng (Singapore)
<b>11:50 AM-12:10 PM</b>	<b><u>SPECIAL LECTURE 2:</u> (15' + 5' discussion)</b> <b>Chair:</b> Tessa Holyoake (Glasgow)	
	Leukemic stem cell interactions with the microenvironment: friend or foe?	Dominique Bonnet (London)
<b>12:10 PM-1:45 PM</b>	<b><i>Lunch and Poster Viewing [Discovery Ballroom &amp; Foyer]</i></b>	
<b>1:45 PM- 3:05 PM</b>	<b><u>SCIENTIFIC SESSION 4: MOLECULAR BIOLOGY AND NEW TARGETS (2)</u></b> <b>Chair:</b> Oliver Hantschel (Lausanne)	
<b>1:45-2:05</b>	<b><u>Keynote Presentation</u> (15' + 5' discussion)</b> Transcriptional control of metabolism in CML and lymphoid blast crisis	Markus Müschen (San Francisco)
	<b><u>Selected Abstracts</u> (12' + 3' discussion)</b>	
2:05-2:20	ABL1 kinase: the frenemy of hematopoietic cells	Yashodhara Dasgupta (Philadelphia)
2:20-2:35	SIRT1 and LSD1 competitively regulate KU70 functions in DNA repair and BCR-ABL mutation acquisition	WenYong Chen (Duarte)
2:35-2:50	Dynamic spectrum of somatic mutations in response to tyrosine kinase inhibitor therapy in chronic myeloid leukemia	Dennis Dong Hwan Kim (Toronto)
2:50-3:05	Alternative telomere lengthening and genomic instability in chronic myeloid leukemia – the potential role of POT1 and RAP1	Tomasz Stoklosa (Warsaw)
<b>3:05 PM-3:35 PM</b>	<b><i>Coffee Break [Legends Ballroom Foyer]</i></b>	
<b>3:35 PM-3:55 PM</b>	<b><u>SPECIAL LECTURE 3:</u> (15' + 5' discussion)</b> <b>Chair :</b> Feyruz Rassool (Baltimore)	
	Noncoding RNAs, myeloid transcription factors, and leukemia	Daniel G. Tenen (Singapore)
<b>3:55 PM-5:30 PM</b>	<b><u>SCIENTIFIC SESSION 5: MECHANISMS OF RESISTANCE</u></b> <b>Chair:</b> Andreas Hochhaus (Jena)	
<b>3:55-4:15</b>	<b><u>Keynote Presentation</u> (15' + 5' discussion)</b> BCR-ABL1 independent TKI resistance	Michael Deininger (Salt Lake City)
	<b><u>Selected Abstracts</u> (12' + 3' discussion)</b>	
4:15-4:30	Resistance mechanisms of the new allosteric inhibitor ABL001	Laura Eadie (Adelaide)
4:30-4:45	Identification of genetic variants associated with imatinib resistance in chronic myeloid leukemia patients by a targeted sequencing approach	Florence Lichou (Bordeaux)
4:45-5:00	ABCC6 plays a significant role in the transport of nilotinib in both cells lines and primary patient cells, and may contribute to resistance	Laura Eadie (Adelaide)

5:00-5:15	Clinical significance of trisomy 8 that emerges during therapy in chronic myeloid leukemia	Wei Wang (Houston)
5:15-5:30	Mechanism of oncogene addiction in CML	Mohammad Azam (Cincinnati)

**5:30 PM-7:30 PM**

**Poster Walk 1 and Refreshments** [Discovery Ballroom & Foyer]

**Biology Poster Walk Leaders:** Anna Eiring (Salt Lake City) and G. Vignir Helgason (Glasgow)  
**Clinical Poster Walk Leaders:** Dragana Milojkovic (London) and Javier Pinilla-Ibarz (Tampa)

## Saturday, September 17, 2016

**7:30 AM-8:45 AM**

**Continental Breakfast and Poster Viewing** [Discovery Ballroom & Foyer]

**8:30 AM-11:00 AM**

**CLINICAL SYMPOSIUM ON MODERN DIAGNOSTICS** [Legends Ballroom]

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

8:30-8:40	Welcome and Introduction	
8:40-8:55	The role of cytogenetic monitoring in CML management in 2016	Jorge Cortes (Houston)
8:55-9:05	Discussion	
9:05-9:20	Molecular monitoring in CML – getting to a safe place	Martin Müller (Mannheim)
9:20-9:30	Discussion	
9:30-9:45	Molecular monitoring in the context of TFR	Susanne Saussele (Mannheim)
9:45-9:55	Discussion	
9:55-10:10	Mutation screening – what is standard of care today and the role of new modalities	Simona Soverini (Bologna)
10:10-10:20	Discussion	
10:20-10:35	Biomarkers and response monitoring beyond PCR	Deborah White (Adelaide)
10:35-10:45	Discussion	
10:45-11:00	Debate: Is monitoring as recommended by current guidelines applicable and necessary in standard care of patients with CML	<b>Pro:</b> Giuseppe Saglio (Turin) <b>Con:</b> Charles Schiffer (Detroit)

**9:00 AM-11:00 AM****WORKSHOP FOR NON-CLINICAL SCIENTISTS #2** *[Founders Ballroom I-II]***HUMANIZED MOUSE MODELS****Chairs:** Ravi Bhatia (Birmingham), Brian Huntly (Cambridge)

9:00-9:05	Introduction	
9:05-9:30	Humanized mice to study hematopoiesis and immunity in health and disease	Anthony Rongvaux (Seattle)
9:30-9:40	Discussion	
9:40-10:05	Coinjection of human mesenchymal cells to enhance human cell engraftment	Hind Medyouf (Frankfurt)
10:05-10:15	Discussion	
10:15-10:40	Using mutant kit to enhance human HSC engraftment in mice	Claudia Waskow (Dresden)
10:40-10:50	Discussion	
10:50-11:00	Concluding Remarks	

**11:00 AM-11:30 AM***Coffee Break [Legends Ballroom Foyer]***11:30 AM-11:58 PM****BRIEF ORAL COMMUNICATIONS (BIOLOGY)** *(4 slides/5' + 2' discussion)***Chair:** Tomasz Skorski (Philadelphia)

11:30-11:37	Histone deacetylase inhibitors modulate DSB repair in leukemias by facilitating increased PARP1 entrapment to DSBs in chromatin	Pratik Nagaria (Baltimore)
11:37-11:44	Anti-tumor efficacy of arsenic/interferon in preclinical models of chronic myeloid leukemia resistant to tyrosine kinase inhibitors	Rihab Nasr (Beirut)
11:44-11:51	Tunneling nanotube communication in tyrosine kinase inhibitor treated chronic myeloid leukemia cells	Maria Omsland (Bergen)
11:51-11:58	Novel hot spot mutations in BCR-ABL1: are we looking the right way to TKI resistance?	Sabrina Pricl (Trieste)

**11:58 PM-12:26 PM****BRIEF ORAL COMMUNICATIONS (CLINICAL)** *(4 slides/5' + 2' discussion)***Chair:** Stuart Goldberg (Hackensack)

11:58-12:05	Second generation TKIs provide a more effective treatment with higher persistence rates than imatinib in "real-world" newly diagnosed CML patients	Inge G.P. Geelen (Dordrecht)
12:05-12:12	The dynamics of CML prevalence in France, from relative survival and demographic data. History and forecast for the next decades	Marc Delord (Paris)
12:12-12:19	Implementing a certified plasmid reference material for copy number calibration demonstrates good reporting correlation between %BCR-ABL/ABL (IS) and %BCR-ABL/ABL (Copy Number) in Xpert® BCR-ABL Ultra	Gwo-Jen Day (Sunnyvale)
12:19-12:26	Adjuvant immunotherapy results in durable TKI-free responses in CML-CP patients	Jonathan Webster (Baltimore)

12:30 PM-2:30 PM

Lunch and Poster Viewing [Discovery Ballroom & Foyer]

1:00 PM-2:30 PM

**MID-DAY SATELLITE SYMPOSIUM**

**6<sup>th</sup> Classical MPN Colloquium at the John Goldman Conference on CML**

**Chair:** Srdan Verstovsek (Houston)

Introduction

Tariq Mughal (Boston)

Genetics and pathogenesis of BCR-ABL1-negative MPNs

Michael Deininger  
(Salt Lake City)

Diagnostic and treatment enigmas in polycythemia vera

Richard Silver (New York)

Current and future treatment strategies for myelofibrosis

Raajit Rampal (New York)

Topical challenges in the management of essential  
thrombocythemia

Srdan Verstovsek  
(Houston)

Clonal evolution of MPNs to AML and some therapeutic  
insights

Farhad Ravandi-Kashani  
(Houston)

Panel discussion & Closing remarks

Srdan Verstovsek  
(Houston)

*Panelists :* Jerry Radich (Seattle); Robyn Scherber (Portland);  
Jerry Spivak (Baltimore); Toyosi Odenyike (Chicago); and faculty

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

2:30 PM-3:35 PM

**SCIENTIFIC SESSION 6: BIOMARKERS**

**Chair:** Deborah White (Adelaide)

2:30-2:50

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

Biomarkers for prognostic evaluation: how to identify the best ? François Guilhot (Poitiers)

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

2:50-3:05

BIM deletion polymorphism as a prognostic biomarker in  
conjunction with the EUTOS long-term survival (ELTS) score in  
Asian CML patients treated with imatinib

Hein Than (Singapore)

3:05-3:20

Reduced CD62L expression on T cells and increased soluble  
CD62L levels predict molecular response to nilotinib therapy  
early chronic phase chronic myelogenous leukemia (CML-CP)

Dominik Wolf (Bonn)

3:20-3:35

Single-cell analysis reveals stem-cell program of CML stem cells

Chinmay Rajiv Munje (Glasgow)

3:35 PM-4:05 PM

**Coffee Break** [Legends Ballroom Foyer]

4:05 PM-4:25 PM

**SPECIAL LECTURE 4:** (15' + 5' discussion)

**Chair:** David Scheinberg (New York)

Towards identification and targeting of leukemic stem cells  
using humanized niche xenograft mouse models

Jan Jacob Schuringa (Groningen)

4:25 PM-5:45 PM

**SCIENTIFIC SESSION 7: THERAPEUTIC INTERVENTIONS**

**Chair:** François-Xavier Mahon (Bordeaux)

4:25-4:45

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

From imatinib to...imatinib: CML treatment comes home,  
via stem cells and fatigue

Carlo Gambacorti-Passerini (Monza)

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

4:45-5:00

BP1001, a novel therapeutic for chronic myelogenous leukemia

Ana Tari Ashizawa (Bellaire)

5:00-5:15

Long-term efficacy and safety of ponatinib in heavily pretreated  
leukemia patients: 4-year results from the pivotal phase 2  
PACE trial

Jorge Cortes (Houston)

5:15-5:30

Efficacy and safety following bosutinib dose reduction in patients  
with philadelphia chromosome-positive chronic myeloid leukemia

Vamsi Kota (Atlanta)

5:30-5:45

Interferon alpha salvages recurrent or persisting post-transplant  
relapse of CML refractory to DLI and TKI producing sustained  
molecular remissions and operational cures

John Barrett (Bethesda)

5:45 PM-5:55 PM

**JOHN GOLDMAN PRIZE PRESENTATION**

Jorge Cortes (Houston)

**Awardee 2016:** Hagop Kantarjian (Houston)

5:55 PM-6:15 PM

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

**Chair:** Jorge Cortes (Houston)

CML Beyond 2016 – Some important questions

Hagop Kantarjian (Houston)

6:15 PM-7 :15 PM

***Poster Walk 2 and Refreshments [Discovery Ballroom & Foyer]***

**Biology Poster Walk Leaders:** Anna Eiring (Salt Lake City) and G. Vignir Helgason (Glasgow)

**Clinical Poster Walk Leaders:** Dragana Milojkovic (London) and Javier Pinilla-Ibarz (Tampa)

**Sunday, September 18, 2016**

7:00 AM-8:00 AM

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

*Get your day off to a great start! Join us for an early 5km run at Houston's beautiful Memorial Park. Shuttles will leave the Royal Sonesta Hotel starting at 6:30 AM and will return from Memorial Park starting at 7:30 AM.*

7:30 AM-8:45 AM

***Continental Breakfast and Poster Viewing [Discovery Ballroom & Foyer]***

8:30 AM-10:05 AM

**SCIENTIFIC SESSION 8: PREDICTORS AND MODELING OF RESPONSE**

**Chair:** Javier Pinilla-Ibarz (Tampa)

8:30-8:50

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

Linking disease response to clinical outcomes in CML:  
Where are we ?

Pierre Laneuville (Montreal)



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

8:50-9:05	Impact of early responses on 3-year outcomes in heavily pretreated CP-CML patients: landmark analyses in the pivotal ponatinib PACE trial	Martin C. Müller (Mannheim)
9:05-9:20	Acceptable molecular response of BCR-ABL levels in patients with chronic myeloid leukemia in chronic phase	Koji Sasaki (Houston)
9:20-9:35	Early response at 3 months with radotinib or imatinib in chronic myeloid leukemia from a randomized phase 3 trial (RERISE)	Young Rok Do (Daegu)
9:35-9:50	Predictors of stable deep molecular response in chronic phase CML patients treated with dasatinib or nilotinib after imatinib failure	Massimiliano Bonifacio (Verona)
9:50-10:05	Prognostic significance of treatment and response duration for successful stopping tyrosine kinase inhibitors chronic myeloid leukemia patients: results of the EURO-SKI trial	Susanne Saussele (Mannheim)

**10:05 AM-11:10 AM**

**SCIENTIFIC SESSION 9: SAFETY, COST AND EXPERIENCE**

**Chair:** François-Xavier Mahon (Bordeaux)

**10:05-10:25**

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

Choosing a therapy for CML – what to consider when you actually have options

Jeffrey Lipton (Toronto)

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

10:25-10:40	Higher out-of-pocket expenses for tyrosine kinase inhibitor therapy is associated with worse health-related quality-of-life in persons with chronic myeloid leukemia	Qian Jiang (Beijing)
10:40-10:55	CML treatment in Dutch hospitals with low patient volumes is associated with a substandard quality of molecular response monitoring	Inge G.P. Geelen (Dordrecht)
10:55-11:10	Arterio-thrombotic events among chronic myeloid leukemia patients on tyrosine kinase inhibitors	Hycienth Ahaneku (Houston)

**11:10 PM-11:20 PM**

**iCMLf PRIZE PRESENTATION:**

**2016 Awardee:** Susan Branford (Adelaide)

Tim P. Hughes (Adelaide)

**11:20 PM-11:40 PM**

**SPECIAL LECTURE 6:** (15' + 5' discussion)

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

Challenges for monitoring patients in emerging economic regions

Susan Branford (Adelaide)

**11:40 AM-12:10 PM**

**DEBATE:** TFR ATTEMPTS – MORE DOWNSIDE THAN BENEFITS

**Chair:** Pierre Laneuville (Montreal)

**Pro:** Michael Mauro (New York)

**Con:** Delphine Réa (Paris)

**12:10 PM**

***Meeting Adjourned***