

Primary analysis of a phase II open-label trial of INCB039110, a selective JAK1 inhibitor, in patients with myelofibrosis

John O. Mascarenhas,¹ Moshe Talpaz,² Vikas Gupta,³ Lynda M. Foltz,⁴ Michael R. Savona,⁵ Ronald Paquette,^{6*} A. Robert Turner,⁷ Paul Coughlin,⁸ Elliott Winton,⁹ Timothy C. Burn,¹⁰ Peter O'Neill,¹⁰ Jason Clark,¹⁰ Deborah Hunter,¹⁰ Albert Assad,¹⁰ Ronald Hoffman¹ and Srdan Verstovsek¹¹

¹The Icahn School of Medicine at Mount Sinai, New York, NY, USA; ²University of Michigan Cancer Center, Ann Arbor, MI, USA; ³Princess Margaret Cancer Centre, Toronto, ON, Canada; ⁴St. Paul's Hospital, University of British Columbia, Vancouver, BC, Canada; ⁵Vanderbilt-Ingram Cancer Center, Vanderbilt University Medical Center, Nashville, TN, USA; ⁶University of California, Los Angeles, CA, USA; ⁷Cross Cancer Institute Edmonton, AB, Canada; ⁸Monash University, VIC, Australia; ⁹Winship Cancer Institute, Emory University, Atlanta, GA, USA; ¹⁰Incyte Corporation, Wilmington, DE, USA and ¹¹The University of Texas MD Anderson Cancer Center, Houston, TX, USA

*Current affiliation: Cedars-Sinai, Los Angeles, CA, USA

©2017 Ferrata Storti Foundation. This is an open-access paper. doi:10.3324/haematol.2016.151126

Received: June 23, 2016.

Accepted: October 17, 2016.

Pre-published: October 27, 2016.

Correspondence: john.mascarenhas@mssm.edu

Supplemental Data

Table of Contents

INCB 39110-230 Study Investigators2

Table S1. Infections and infestations by maximum severity in the safety evaluable population..... 3

Table S2. Laboratory value shift tables for anemia by dose cohort4

Table S3. Laboratory value shift tables for thrombocytopenia by dose cohort5

Figure S1. Study design6

Figure S2. PGIC scores over time by dose cohort7

INCB 39110-230 Study Investigators

The following investigators contributed to the study (listed in alphabetical order by country):

Australia—J. V. Catalano, Frankston Hospital and Department of Clinical Haematology, Monash University, Frankston, Melbourne, Victoria; B. H. Chong, St. George Hospital, Kogarah, NSW; P. Coughlin, Monash University/Box Hill Hospital, Box Hill, Victoria; D. Ma, St. Vincent's Hospital, Darlinghurst, NSW. **Canada**—L. Foltz, St. Paul's Hospital, University of British Columbia, Vancouver, BC; V. Gupta, Princess Margaret Hospital, University of Toronto, Toronto, ON; S. Sirhan, Jewish General Hospital, Montreal, QC; R. Turner, Cross Cancer Institute, Edmonton, AB. **United States**—E. O. Hexner, Abramson Cancer Center at the University of Pennsylvania, Philadelphia, PA; J. O. Mascarenhas, Mount Sinai School of Medicine, New York, NY; C. Miller, St. Agnes HealthCare, Inc, Baltimore, MD; R. Paquette, University of California-Los Angeles Medical Hematology and Oncology, Los Angeles, CA; M. Raza, Boston Baskin Cancer Foundation, Inc., Memphis, TN; M. Savona/I. Flinn, Sarah Cannon Research Institute, Nashville, TN; M. Talpaz, University of Michigan Medical Center, Ann Arbor, MI; S. Verstovsek, University of Texas M.D. Anderson Cancer Center, Houston, TX; E. F. Winton, Winship Cancer Institute of Emory University, Emory University Hospital, Atlanta, GA.

Table S1. Infections and infestations by maximum severity in the safety evaluable population.

Preferred term, Number of patients (%)	Mild	Moderate	Severe	Life threatening	Total (N=87)
Abscess oral			1		1 (1.1)
Bronchitis		4			4 (4.6)
Cellulitis		1			1 (1.1)
Ear infection	1				1 (1.1)
Epiglottitis			1		1 (1.1)
Eye infection		1			1 (1.1)
Folliculitis		1			1 (1.1)
Gastroenteritis	1				1 (1.1)
Gastrointestinal viral infection	1				1 (1.1)
Herpes simplex		2			2 (2.3)
Herpes zoster		2			2 (2.3)
Hordeolum		1			1 (1.1)
Infection		1			1 (1.1)
Influenza	1				1 (1.1)
Oral candidiasis	1	1			2 (2.3)
Oral herpes		3			3 (3.4)
Oral viral infection		1			1 (1.1)
Pneumonia	1	1	2	1	5 (5.7)
Sinusitis		4			4 (4.6)
Skin infection		1			1 (1.1)
Tooth abscess		1			1 (1.1)
Tooth infection		1			1 (1.1)
Upper respiratory tract infection	8	9			17 (9.5)
Urinary tract infection	1	4	2		7 (8.0)
Viral infection	2				2 (2.3)

Table S2. Laboratory value shift tables for anemia by dose cohort.

Anemia					
100 mg twice daily					
Baseline (N=10)	<i>Extreme postbaseline value, n</i>				
	<i>Grade 0</i>	<i>Grade 1</i>	<i>Grade 2</i>	<i>Grade 3</i>	<i>Grade 4</i>
Grade 0 (n=0)	0	0	0	0	0
Grade 1 (n=4)	0	1	3	0	0
Grade 2 (n=5)	0	0	2	3	0
Grade 3 (n=1)	0	0	1	0	0
Grade 4 (n=0)	0	0	0	0	0
200 mg twice daily					
Baseline (N=45)	<i>Extreme postbaseline value, n</i>				
	<i>Grade 0</i>	<i>Grade 1</i>	<i>Grade 2</i>	<i>Grade 3</i>	<i>Grade 4</i>
Grade 0 (n=7)	4	2	1	0	0
Grade 1 (n=13)	0	5	6	2	0
Grade 2 (n=22)	0	3	5	14	0
Grade 3 (n=3)	0	0	0	3	0
Grade 4 (n=0)	0	0	0	0	0
600 mg once daily					
Baseline (N=32)	<i>Extreme postbaseline value, n</i>				
	<i>Grade 0</i>	<i>Grade 1</i>	<i>Grade 2</i>	<i>Grade 3</i>	<i>Grade 4</i>
Grade 0 (n=6)	2	3	1	0	0
Grade 1 (n=8)	0	6	1	1	0
Grade 2 (n=18)	0	2	9	7	0
Grade 3 (n=0)	0	0	0	0	0
Grade 4 (n=0)	0	0	0	0	0

Gray cells represent improvement and black cells represent worsening.

Table S3. Laboratory value shift tables for thrombocytopenia by dose cohort

Thrombocytopenia					
100 mg twice daily					
Baseline (N=10)	<i>Extreme postbaseline value, n</i>				
	<i>Grade 0</i>	<i>Grade 1</i>	<i>Grade 2</i>	<i>Grade 3</i>	<i>Grade 4</i>
Grade 0 (n=2)	1	1	0	0	0
Grade 1 (n=4)	0	2	0	2	0
Grade 2 (n=3)	0	1	0	2	0
Grade 3 (n=0)	0	0	0	0	0
Grade 4 (n=1)	0	1	0	0	0
200 mg twice daily					
Baseline (N=45)	<i>Extreme postbaseline value, n</i>				
	<i>Grade 0</i>	<i>Grade 1</i>	<i>Grade 2</i>	<i>Grade 3</i>	<i>Grade 4</i>
Grade 0 (n=22)	9	5	4	4	0
Grade 1 (n=14)	0	4	5	4	1
Grade 2 (n=9)	0	1	1	5	2
Grade 3 (n=0)	0	0	0	0	0
Grade 4 (n=0)	0	0	0	0	0
600 mg once daily					
Baseline (N=32)	<i>Extreme postbaseline value, n</i>				
	<i>Grade 0</i>	<i>Grade 1</i>	<i>Grade 2</i>	<i>Grade 3</i>	<i>Grade 4</i>
Grade 0 (n=22)	14	5	2	1	0
Grade 1 (n=8)	0	2	3	2	1
Grade 2 (n=2)	0	0	1	1	0
Grade 3 (n=0)	0	0	0	0	0
Grade 4 (n=0)	0	0	0	0	0

Gray cells represent improvement and black cells represent worsening.

Figure S1. Study design. TSS indicates total symptom score.

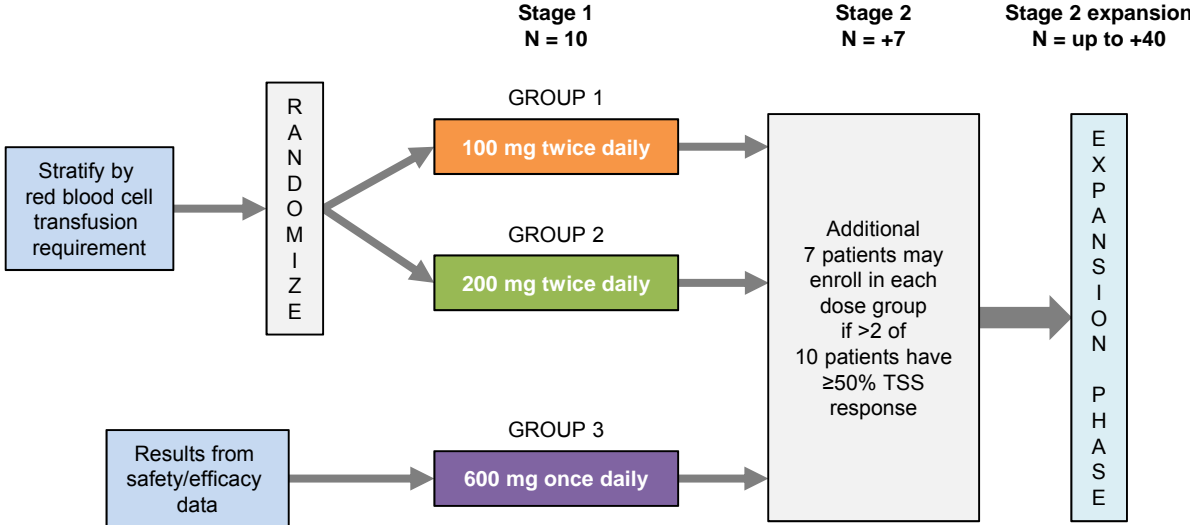


Figure S2. PGIC scores over time by dose cohort. Shown are the proportions of evaluable patients with a score of 1 (very much improved) or 2 (much improved).

