



**ONCONOVA**  
T H E R A P E U T I C S

# Executive Summary

November 2017

Nasdaq: ONTX

## **FORWARD LOOKING STATEMENTS**

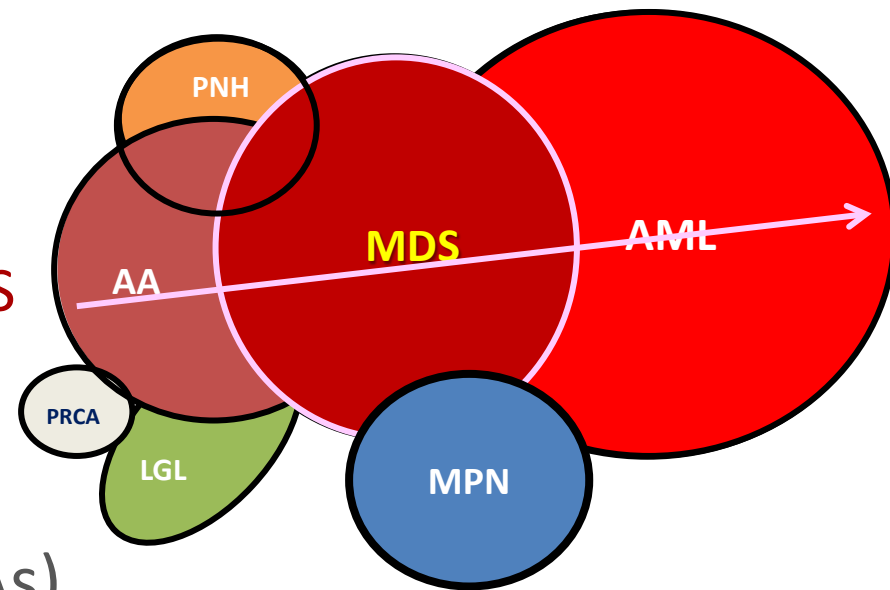
This presentation contains forward-looking statements about Onconova Therapeutics, Inc. based on management's current expectations which are subject to known and unknown uncertainties and risks. Onconova has attempted to identify forward-looking statements by terminology including "believes," "estimates," "anticipates," "expects," "plans," "intends," "may," "could," "might," "should," "approximately" or other words that convey uncertainty of future events or outcomes. Our actual results could differ materially from those discussed due to a number of factors, including, but not limited to, our ability to raise additional financing on favorable terms, the success of our clinical trials and our ability to obtain regulatory approvals and other risk factors outlined in our annual and quarterly reports filed with the Securities and Exchange Commission. We are providing this information as of the date of this presentation and do not undertake any obligation to update any forward-looking statements, whether written or oral, that may be made from time to time, as a result of new information, future events or otherwise except as required by law.

# ONCONOVA HIGHLIGHTS

- Company founded in 1998 and public since 2013 (Nasdaq: ONTX)
  - Targeting underserved needs in Myelodysplastic Syndromes (MDS)
  - Lead drug Rigosertib in Phase 3 “INSPIRE” trial for Higher-risk MDS
    - Currently no approved drugs for 2<sup>nd</sup> line patients
  - Designing Phase 3 trial for Oral rigosertib + azacitidine combination
- Key upcoming milestones
    - INSPIRE (IV) Phase 3 interim analysis expected in **Q4-2017**
    - Full trial enrollment and Top-line Phase 3 data in **2018**
- Actively seeking partnerships
    - Rigosertib licensed to SymBio in Japan; other territories in discussion
    - High value preclinical stage next generation CDK4/6 inhibitor

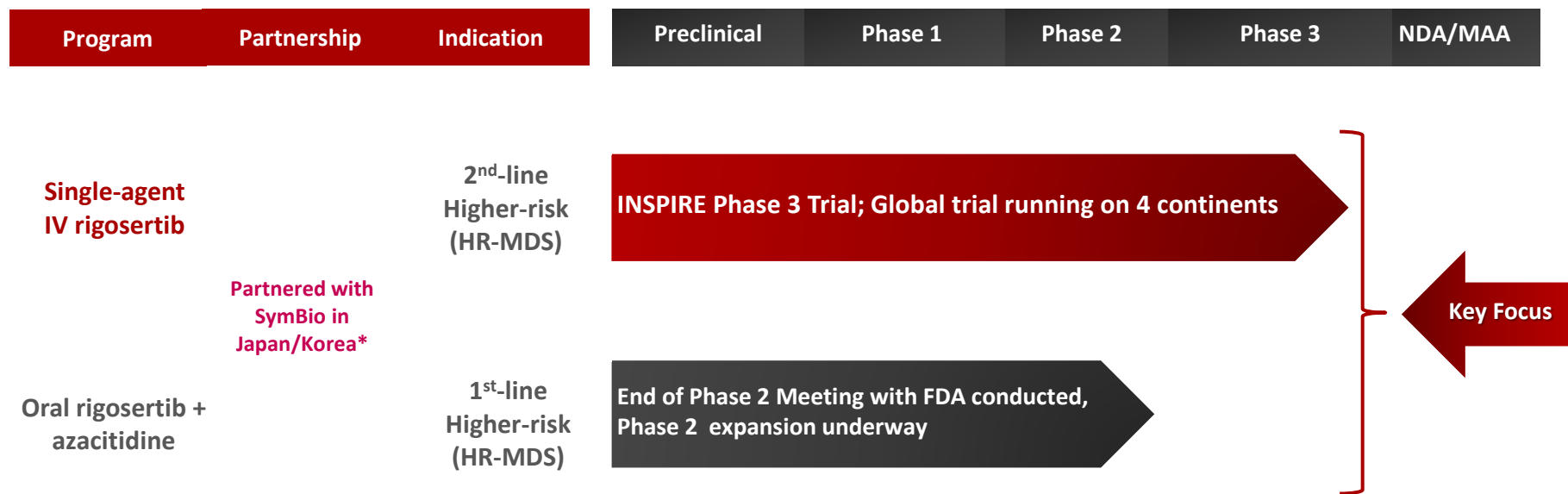
# MDS OVERLAPS WITH OTHER DISEASES

- MDS: malignant bone marrow disorder characterized by:<sup>[1]</sup>
  - Bone marrow failure
  - Low blood counts
  - 30% of patients progress to AML
- US prevalence is 59,000
  - 18,000 have higher risk (HR) MDS
  - ~10,000 second-line patients
- Treatment options limited to hypomethylating agents (HMAs)
  - Vidaza (Celgene); Dacogen (Eisai/J&J)
  - Approved >a decade ago; now off-patent



<sup>1</sup>Young NS. Ann Intern Med. 2002;136:534-546.

# ONCONOVA MDS PIPELINE



- >700 MDS patients have been treated in rigosertib Phase 1-3 trials
  - IV and Oral rigosertib, plus oral rigosertib combination with azacitidine
  - Issued patents and orphan drug designation in US, Europe and Japan

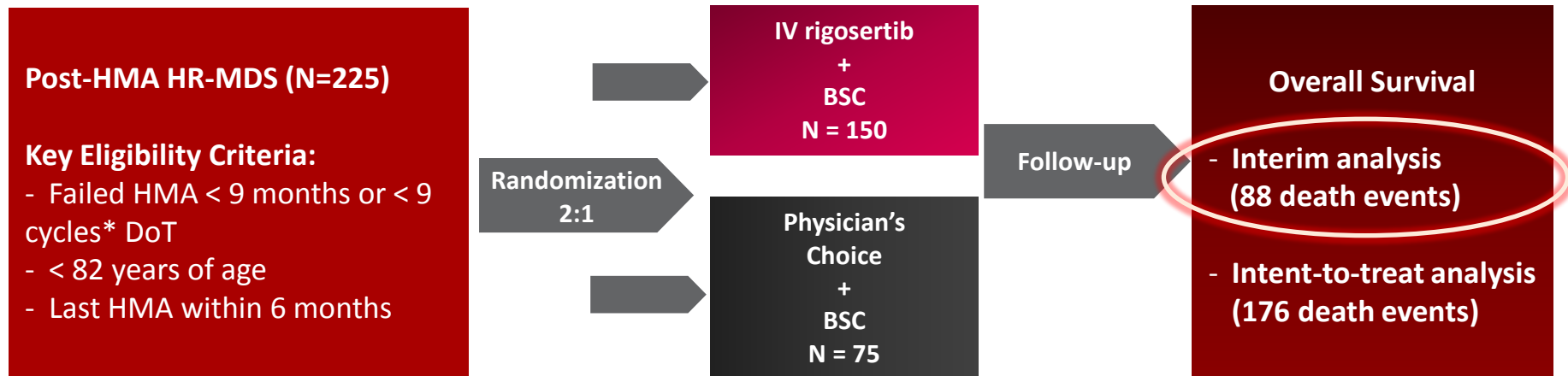


IV product for infusion



Oral soft gel capsules

# INSPIRE TRIAL DESIGN FOR GLOBAL PHASE 3 TRIAL

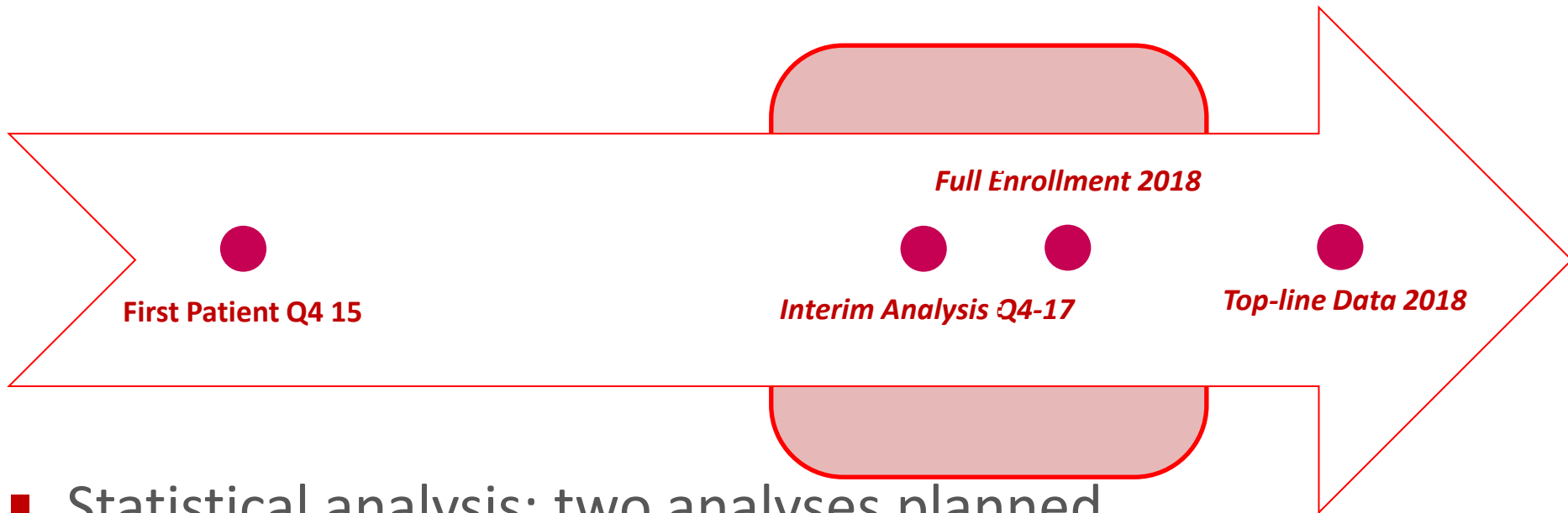


\*9 cycles within 12 months of starting treatment

- Survival endpoint with two successive analyses planned
  - ITT population enriched for higher-risk MDS
  - Second analysis of IPSS-Very High Risk (VHR) predefined group
    - Second cut allows for another chance to succeed in this subpopulation

Commentary on new trial in recent publication: Emilio P Alessandrino, Matteo G Della Porta. Novel trial designs for high-risk myelodysplastic syndromes; *The Lancet Oncology* 2016 (17): 410–412

# TIMELINES FOR DATA ANALYSIS FOR INSPIRE TRIAL

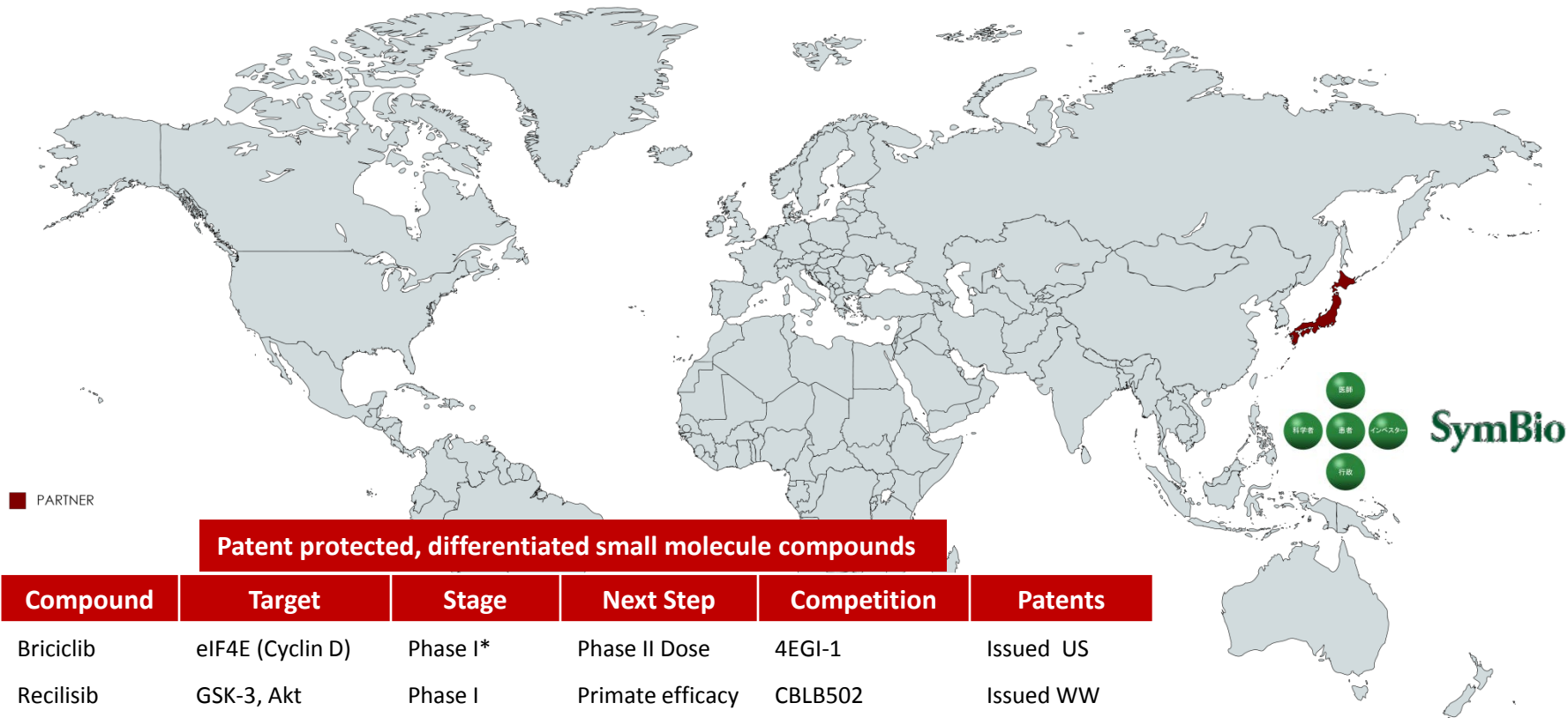


- Statistical analysis: two analyses planned
  - Power 0.80; Target HR < 0.625; (reduce mortality by > 37.5%)
  - $\alpha$  for ITT = 0.04;  $\alpha$  for IPSS-R VHR = 0.01
    - Dual primary endpoints: Overall survival in ITT population or IPSS-R Very High Risk
- Exploratory genomic sequencing of patient samples

# BUSINESS DEVELOPMENT OPPORTUNITIES:

RIGOSERTIB IS PARTNERED IN JAPAN/KOREA SINCE 2011

*Partnerships for pipeline products sought in other territories*



**Patent protected, differentiated small molecule compounds**

Compound	Target	Stage	Next Step	Competition	Patents
Briciclib	eIF4E (Cyclin D)	Phase I*	Phase II Dose	4EGI-1	Issued US
Recilisib	GSK-3, Akt	Phase I	Primate efficacy	CBLB502	Issued WW
<b>ON 123300**</b>	<b>CDK4/6; ARK5</b>	<b>Preclinical</b>	<b>Toxicology</b>	<b>Palbociclib</b>	<b>Issued US, EP</b>
ON 150030**	FLT3 + Src	Pre-clinical	Animal studies	Dasatinib	In process
ON 1231320	PLK2	Formulation	Pre-IND	Volasertib	Issued
ON 108600	CK2	Formulation	Pre-IND	CX-4945	Issued
ON 146040	PI3K a/d	Pre-clinical	Toxicology	IPI-145	In process

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\*On hold, pending new drug product

\*\*New data presented at 2017 AACR conference

# FINANCIAL DETAILS & SUMMARY

**Onconova founded in 1998; public since 2013**

<b>Ticker</b>	Nasdaq ONTX	<b>Debt</b>	\$0
<b>Stock Information</b>	<ul style="list-style-type: none"><li>9.9 million shares</li><li>Public float &gt;84%</li><li>52-week range: \$1.51 - \$3.50</li><li>52-week average daily volume: 117,000</li><li>Oct-2017 average daily volume: 348,000</li></ul>	<b>Liquidity</b>	<ul style="list-style-type: none"><li>Cash and cash equivalents of \$15 million as of 6-30-2017</li><li>Funded to deliver key milestones in 2017</li></ul>
<b>Ownership</b>	Tyndall, Tavistock, Sabby, Shire; insiders including management	<b>Burn-rate</b>	Average \$5.6 million per quarter over the last 5 quarters
<b>Analyst Coverage</b>	H.C. Wainwright, Laidlaw, Maxim, LifeSci Capital, Van Leeuwenhoeck Research (VLR), SeeThru Equity, Dawson James	<b>Partnerships</b>	Rigosertib is partnered with Symbio Pharmaceuticals in Japan/Korea; Onconova retains rights to the rest of the world

- Advanced clinical trials
  - Phase 3 underway (IV rigosertib)
- Funded to deliver key 2017 milestones
  - IV Phase 3 interim analysis Q4-2017; top-line data 2018
- Underserved and growing market in MDS
  - >10,000 patients diagnosed annually
  - No new approved therapies in over a decade
- Rigosertib + pipeline present business development opportunities
- Seasoned management team and board of directors