



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

# Executive Summary

December 2017

Nasdaq: ONTX

## **FORWARD LOOKING STATEMENTS**

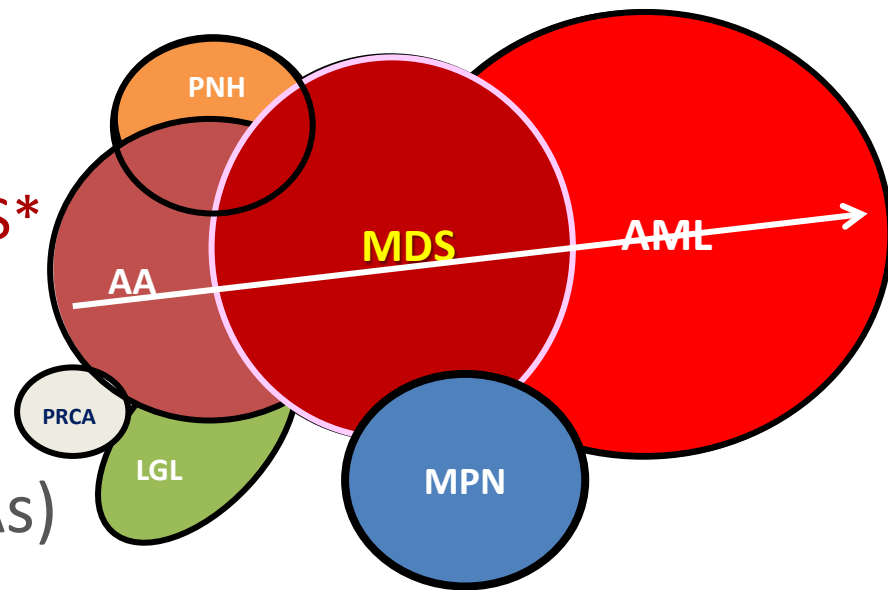
This presentation contains forward-looking statements about Onconova Therapeutics, Inc. based on management's current expectations which are subject to known and unknown uncertainties and risks. Onconova has attempted to identify forward-looking statements by terminology including "believes," "estimates," "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 AT A GLANCE

- Founded in 1998; IPO in 2013 (Nasdaq: ONTX)
- **Phase 3 stage clinical candidate: rigosertib**
  - Targets RAS effector pathways (Cell, 2016)\*
  - Focused on Myelodysplastic Syndromes (MDS)
- Rigosertib partnered with Symbio in Japan/Korea
  - Additional partnerships sought
- Broad pipeline with earlier stage drug candidates

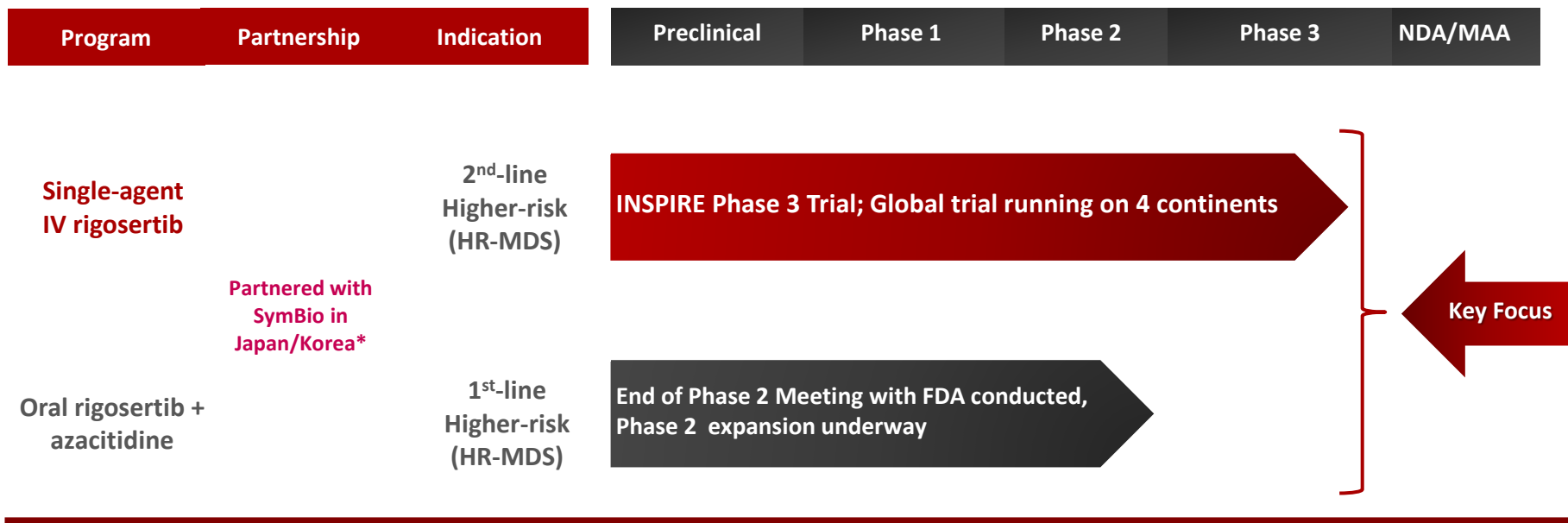
# MDS OVERLAPS WITH OTHER DISEASES

- MDS: malignant bone marrow disorder characterized by:<sup>[1]</sup>
  - Bone marrow failure leading to 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

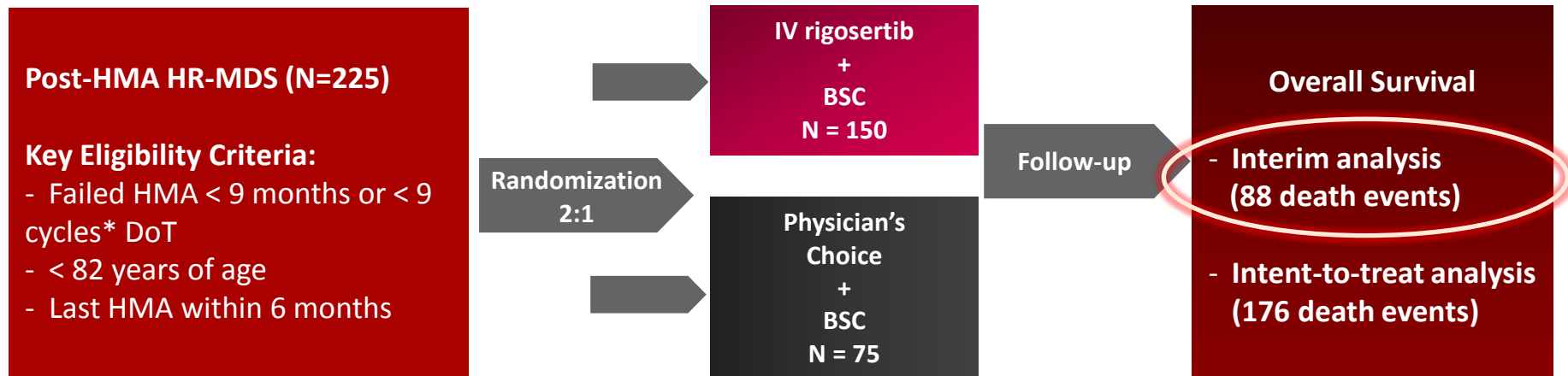


IV product for infusion



Oral soft gel capsules

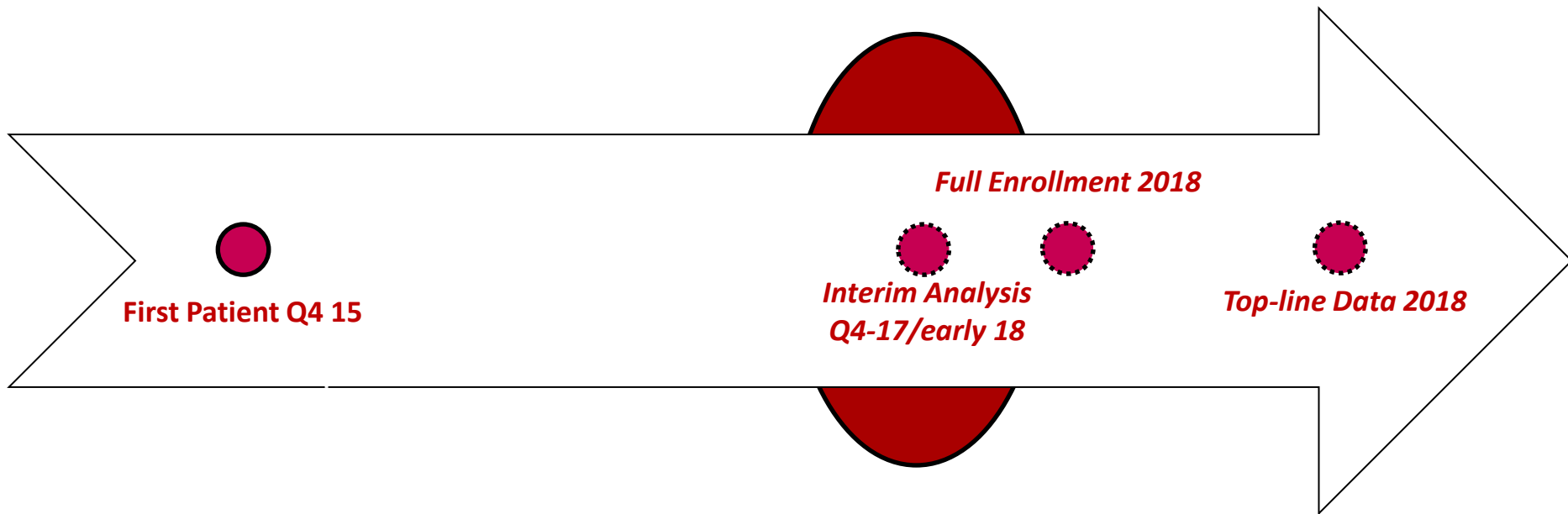
# INSPIRE TRIAL DESIGN FOR GLOBAL PHASE 3 TRIAL



\*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

# TIMELINES FOR DATA ANALYSIS FOR INSPIRE TRIAL



- Statistical analysis plan: two survival 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
    - **Two endpoints: OS 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*



■ PARTNER

**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

*\*On hold, pending new drug product*

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# FINANCIAL DETAILS & SUMMARY

## Onconova founded in 1998; public since 2013

### Ticker

Nasdaq ONTX

### Stock Information

- 10.8 million shares outstanding
- Public float >84%
- 52-week range: \$1.51 - \$3.22
- 52-week average daily volume: 116,000
- 4Q17 average daily volume: 223,000

### Ownership

Tyndall, Tavistock, Sabby, Shire; insiders including management

### Analyst Coverage\*

H.C. Wainwright, Laidlaw, Maxim, LifeSci Capital, Van Leeuwenhoeck Research (VLR). SeeThru Equity, Dawson James

### Debt

\$0

### Liquidity

- Cash and cash equivalents of \$7.6 million as of 9-30-2017 (excluding Nov-17 raise of \$1.4 million)
- Funded to deliver key milestones in 2017

### Burn-rate

Average \$5.6 million per quarter over the last 5 quarters

### Partnerships

Rigosertib is partnered with SymBio Pharmaceuticals in Japan/Korea; Onconova retains rights to the rest of the world

*\*Reports available upon request*