

# **Executive Summary**

September 2017

Nasdaq: ONTX

#### FORWARD LOOKING STATEMENTS

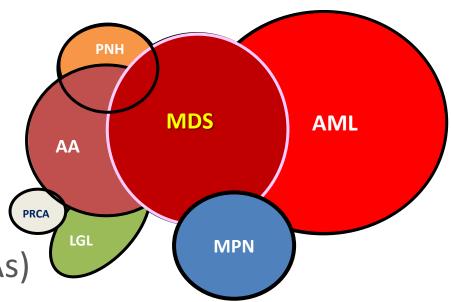
This presentation contains forward-looking statements about Onconova Therapeutics, Inc. based on management's current expectations which are subject to known and unknown uncertainties and risks. Onconova has attempted to identify forward-looking statements by terminology including "believes," "estimates," "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)
- Phase 3 Trial (INSPIRE) running on 4 Continents for 2nd line HR-MDS
- Patents & Orphan Designation for MDS in the US, Europe and Japan
- Designing Phase 3 trial for Oral rigosertib + azacitidine combination
  - Targeting larger first-line patient population for higher risk MDS
- Collaborative program in RASopathies, rare diseases of children
- Key upcoming milestones
  - INSPIRE (IV) Phase 3 interim analysis expected in Q4-2017
  - Full trial enrollment and Top-line Phase 3 data in 2018
- Pipeline assets beyond rigosertib available for partnerships
  - Includes preclinical stage next generation CDK4/6 inhibitor

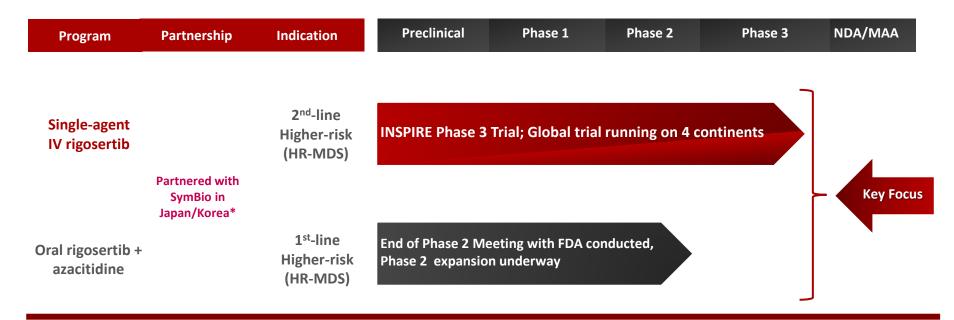
## MDS OVERLAPS WITH OTHER DISEASES

- MDS, a malignant hematopoietic disorder characterized by:<sup>[1]</sup>
  - Bone marrow failure
  - Cytopenias
  - 30% of patients progress to AML
- US prevalence is 59,000
  - 18,000 have higher risk MDS
- Treatment options limited to hypomethylating agents (HMAs)
  - Vidaza (Celgene); Dacogen (Eisai/J&J)
  - Both approved a decade ago



<sup>&</sup>lt;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
  - First and Second-line higher Risk MDS; Lower Risk, transfusion dependent MDS

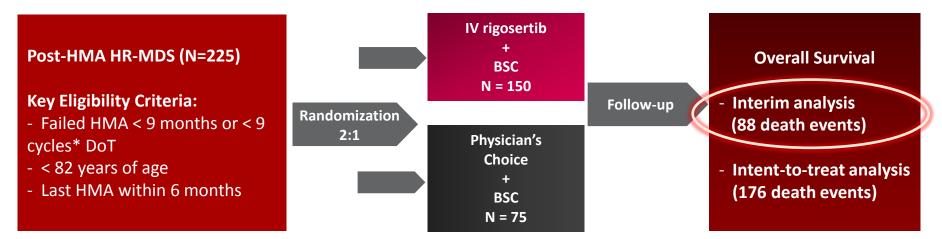


IV product for infusion



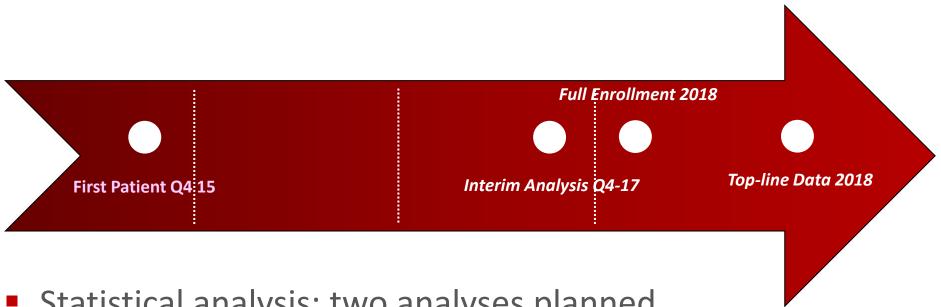
Oral soft gel capsules

#### INSPIRE STUDY DESIGN FOR GLOBAL PHASE 3 TRIAL



- \*9 cycles within 12 months of starting treatment
  - Interim analysis is expected to occur in Q4-2017
  - 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

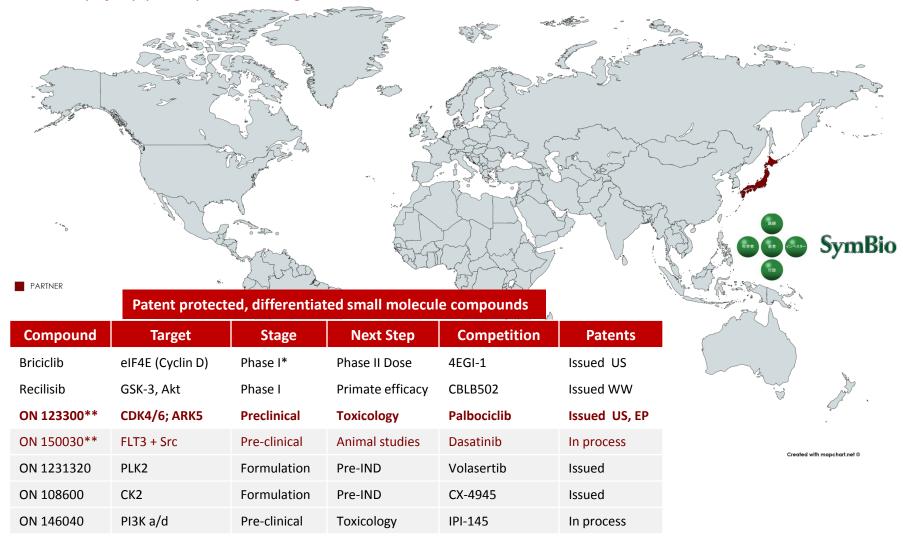


- Statistical analysis: two analyses planned
  - Power 0.80; Target HR < 0.625; (reduce mortality by > 37.5%)
  - $\alpha$  for ITT = 0.04;  $\alpha$  for IPSS-R VHR = 0.01
    - Dual primary endpoints: Overall survival in ITT population or IPSS-R Very High Risk
- Exploratory genomic sequencing of patient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

## **BUSINESS DEVELOPMENT OPPORTUNITIES:**

#### RIGOSERTIB IS PARTNERED IN JAPAN/KOREA SINCE 2011

Partnerships for pipeline products sought in other territories



<sup>\*</sup>On hold, pending new drug product

<sup>\*\*</sup>New data presented at 2017 AACR conference

#### FINANCIAL DETAILS & SUMMARY

Onconova founded in 1998; public since 2013			
Ticker	Nasdaq ONTX	Debt	\$0
Stock Information	<ul> <li>9.9 million shares</li> <li>Public float &gt;84%</li> <li>52-week range: \$1.56 - \$3.50</li> <li>Average daily volume: 91,000</li> </ul>	Liquidity	<ul> <li>Cash and cash equivalents of \$15 million as of 6-30-2017</li> <li>Funded to deliver key milestones in 2017</li> </ul>
Ownership	Tyndall, Tavistock, Sabby, Shire; insiders including management	Burn-rate	Average \$5.6 million per quarter over the last 5 quarters
Analyst Coverage	Laidlaw, Maxim, LifeSci Capital, Van Leeuwenhoeck Research (VLR), SeeThru Equity, Dawson James	Partnerships	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
  - Oral Phase 2 ready to enter Phase 3 trial with additional funding
  - 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
- Preclinical pipeline presents business development opportunities
- Seasoned management team and board of directors