

# Onconova Therapeutics (Nasdaq: ONTX)

**Share Price** (5/01/17) : **\$2.21**  
**Shares Outstanding** (5/01/17) : **9.5M**

Onconova Therapeutics, Inc. is a Phase 3-stage biopharmaceutical company focused on discovering and developing novel small molecule drug candidates to treat cancer. Rigosertib, Onconova's lead candidate, is a proprietary Phase 3-stage small molecule which blocks cellular signaling by targeting RAS effector pathways. The initial focus for rigosertib development is myelodysplastic syndromes (MDS), a group of bone marrow disorders that often converts into acute myeloid leukemia.

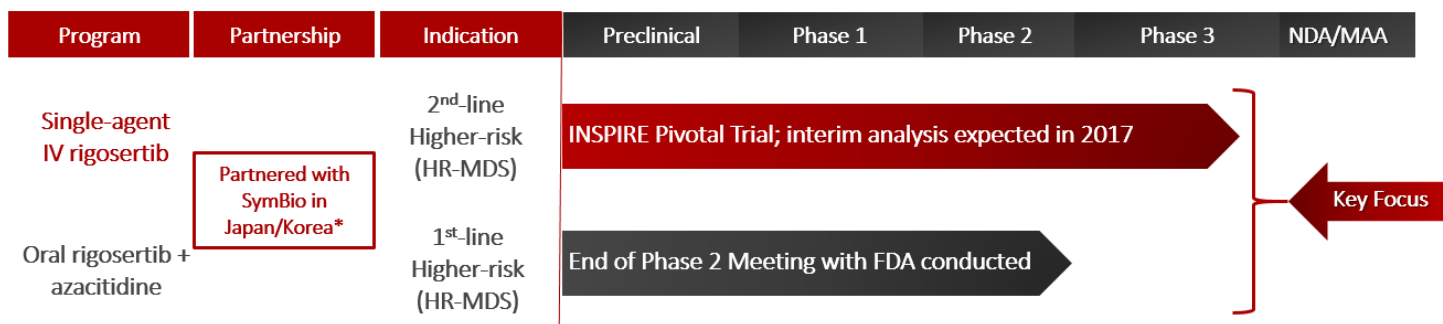
Using a proprietary chemistry platform, Onconova has created a pipeline of targeted anti-cancer agents designed to work against specific cellular pathways that are important in cancer cells, while causing minimal damage to normal cells. Onconova has three product candidates in clinical trials and several active preclinical programs. Advanced clinical trials with our lead compound, rigosertib, are aimed at unmet medical needs of patients with MDS.

## Onconova's Primary Area of Focus: Myelodysplastic Syndromes

MDS is a difficult-to-treat cancer resulting from a hematological malignancy that begins in the bone marrow and influences adequate production of peripheral blood cells. MDS is classified according to severity, from very low risk to very high risk. The illness frequently appears initially as anemia, and can rapidly progress to more advanced disease and, ultimately, to acute myeloid leukemia (AML) in roughly 30% of patients.

For some younger patients, bone marrow transplantation is considered. However, as most patients with MDS are over the age of 65, bone marrow transplantation is generally not a treatment option due to the lengthy time to find a match, and the risks associated with transplantation. While bone marrow transplants and better supportive care have resulted in an improved quality of life for some MDS patients, there have been no advancements in the treatment of MDS in more than a decade. Onconova is currently developing rigosertib as a novel therapeutic treatment for this urgent unmet medical need. MDS overlaps with other hematological disorders.

## Onconova's Cancer Product Pipeline



### Global & regional partnership opportunities

IV Briciclib	Solid tumors	Phase 1 Trial**	eIF4E targeting
ON 123300	CDK4/6 overactive tumors	Pre-IND Stage	ARK5+CDK4/6 targeting

\*Onconova retains rights elsewhere, including USA

\*\*Trial on hold pending partnering and manufacturing of new product lot

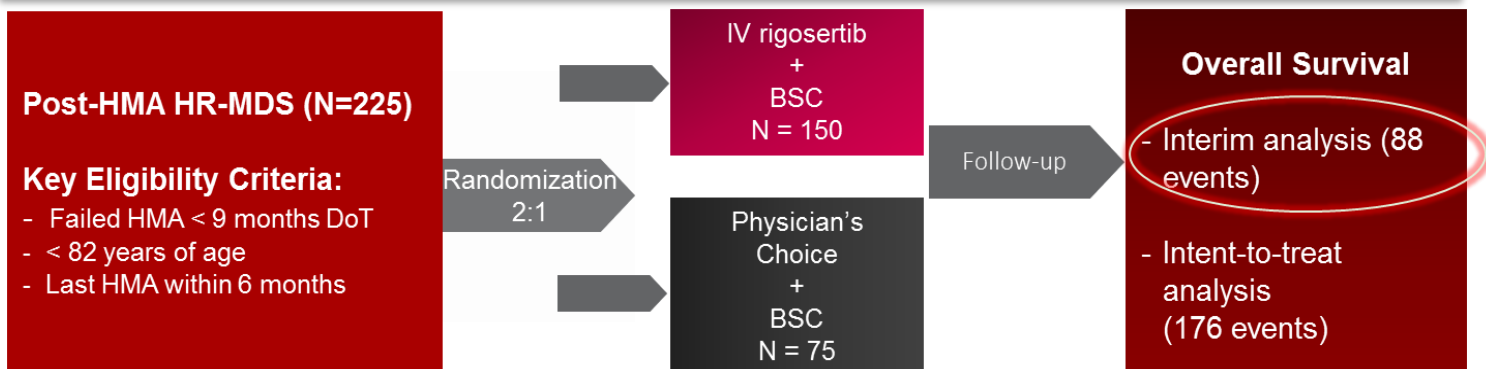
## Onconova Investment Highlights

- Pivotal Phase 3 trial INSPIRE on four continents for small molecule lead drug candidate rigosertib, which targets the RAS effector pathways, one of the most sought after targets in oncology.
- Despite incidence of over 18,000 patients, no approvals in MDS in more than a decade. Current options are not curative and carry debilitating side effects.
- INSPIRE trial is focused on sub-population of patients where the drug was highly effective and identified in the previously completed ONTIME Phase 3 study.
- Rigosertib is protected by issued patents and Orphan Designation for MDS in the U.S., Europe, and Japan.
- Oral rigosertib, in combination with azacitidine, is being developed for larger 1st line patient population, with a Phase 3 trial expected to launch in 2H 2017.
- Broad pipeline with clinical and several pre-clinical stage drug candidates.
- Seasoned management team and Board with established track record in biotech, finance and large pharma.

## Lead Program - INSPIRE Trial

A Phase 3, international, randomized, controlled study of rigosertib versus physician's choice of treatment in patients with higher risk myelodysplastic syndrome after failure of a hypomethylating agent.

The primary objective of this study is to compare the overall survival (OS) of patients receiving intravenous (IV) rigosertib to the OS of patients receiving the physician's choice of treatment (PC) in a population of patients with myelodysplastic syndromes (MDS) after failure of treatment with a hypomethylating agent (HMA), azacitidine (AZA) or decitabine (DEC).



Statistical analysis, two analyses planned:

- $\alpha$  for ITT = 0.04;
- $\alpha$  for IPSS-R VHR = 0.01

Trial can succeed in two ways: ITT population or IPSS-R Very High Risk

## Secondary Program - Combination Oral Rigosertib + Azacitidine

Following evaluation of 54 enrolled patients in the Phase 2 Trial 09-08 of oral rigosertib plus azacitidine, Onconova conducted an End-of-Phase 2 meeting during which updated results from this trial were discussed with the FDA. Based on these discussions, and guidance from the Agency, Onconova will design a randomized, controlled Phase 3 clinical trial comparing the combination of oral rigosertib plus azacitidine to azacitidine plus placebo in hypomethylating agent (HMA) naïve HR-MDS patients.

## Current Financial Highlights

- Oversubscribed rights offering in July 2016 with gross proceeds of \$17.4 million.
- Ownership includes: Tyndall, Tavistock, Sabby, Shire, and insiders including management.
- Analyst coverage includes: Maxim, Van Leeuwenhoeck Research (VLR), SeeThru Equity, LifeSci Advisors.
- Rigosertib is partnered with SymBio Pharmaceuticals in Japan/Korea; Onconova is also exploring other partnership opportunities.
- No debt and cash equivalents of \$25.8 million (as per Q3 financials).

## Contact Information

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## Management Team

### Ramesh Kumar, Ph.D.

*President & CEO, Co-founder*

### Steven M. Fruchtman, M.D.

*Chief Medical Officer*

### Mark Guerin

*Chief Financial Officer*

### Manoj Maniar, Ph.D.

*Senior VP, Product Development*

### Wolfgang Meyer, Ph.D.

*Sr. VP Regulatory Affairs; GM, Onconova GmbH*

### Michael Petrone, M.D.

*Sr. VP Clin. Dev. Medical Affairs & Pharmacovigilance*



# ONCONOVA

TARGETING CANCER | PROTECTING HEALTHY CELLS

Or visit <http://investor.onconova.com/index.cfm>