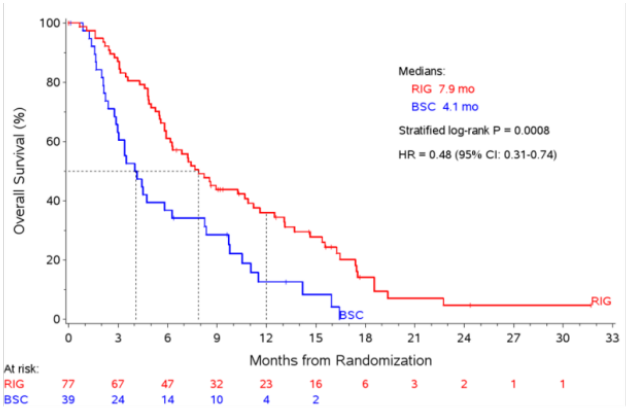
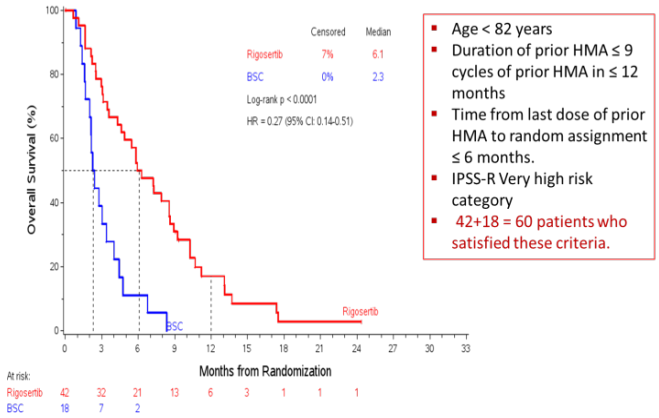
- Discovering and developing novel small molecule drugs, with an initial focus on underserved needs in Myelodysplastic Syndromes (MDS)
- Founded in 1998; Publicly traded (Nasdaq: ONTX) since 2013
- Phase 3 "INSPIRE" trial with IV rigosertib for Higher-Risk MDS patients after failure of front-line therapies is progressing after promising interim analysis, with over 75% enrollment. Full enrollment projected in 2H2019. Currently, there are no approved drugs for 2nd line patients with MDS.
- Protocol and special protocol assessment (SPA) for Phase 3 trial for oral rigosertib + azacitidine combination for front-line indications in MDS submitted to FDA in Dec-18, with ongoing protocol finalization based on FDA feedback.
- Rigosertib is patent-protected until 2037 and has orphan designation for MDS.
- Pipeline of targeted anti-cancer agents, including a CDK4/CDK6 + ARK5 inhibitor ON 123300

LEAD CANDIDATE RIGOSERTIB

INSPIRE randomized controlled pivotal trial with IV rigosertib for 360 patients, with 2:1 randomization, rigosertib treatment group vs. physician's choice. Endpoint is overall survival, 288 events.

INSPIRE is based on learnings from prior trial of 299 patients:



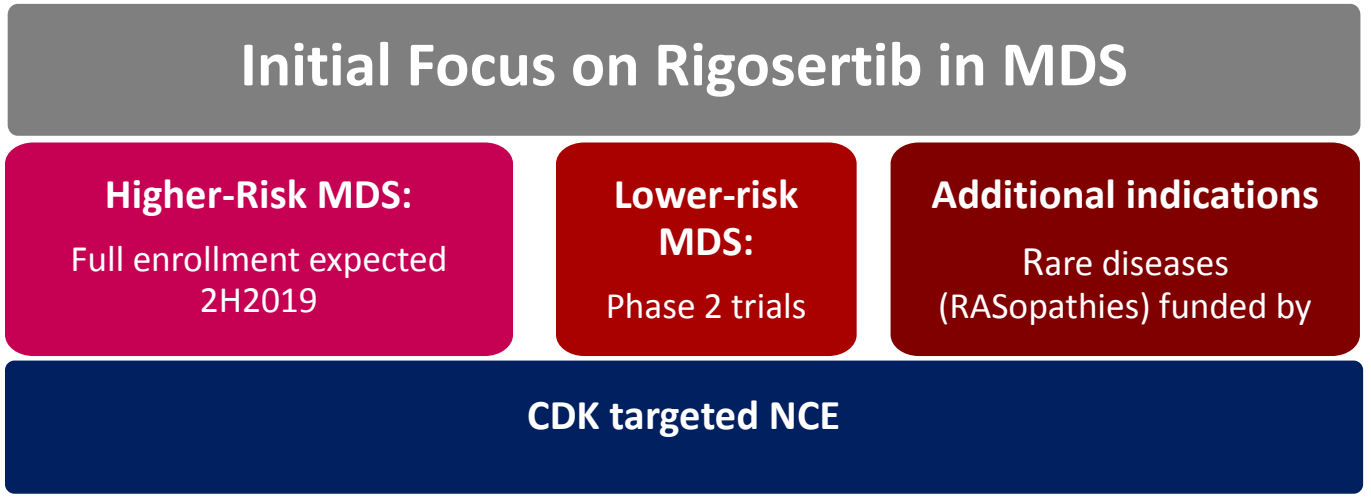


The oral formulation of rigosertib in combination with Azacitidine, the current standard of care, has completed Phase 2 trials in front-line Higher-Risk MDS patients. A randomized trial in first-line Higher-Risk MDS patients is planned in which oral rigosertib + azacitidine will be compared to azacitidine + placebo.

Evaluable for response	29*
Overall response per IWG 2006	26 (90%)
CR+PR	10 (34%)
Complete remission (CR) Partial remission (PR) Marrow CR + Hematologic Improvement Hematologic Improvement alone Marrow CR alone Stable disease Progression	10 (34%) 0 5 (17%) 3 (10%) 8 (28%) 3 (10%) 0
Median duration of response (months)	12.2 (range, 0.1-24.2+)
Median duration of treatment (months)	7.8 (range, 0.7-25.1+)
Median time to initial/best response (cycles)	1/4

** Includes 2 patients treated with non-HMA, prior chemotherapy*

Navada et al: *Blood* 2018 132:230



ONTX LICENSING OPPORTUNITIES

Patent-protected, differentiated small molecule compounds

Compound	Target	Stage	Next Step	Other Agents	Patents	Licensing Territories Available
			Clinical Stage			
Rigosertib	▪ RAS pathway ▪ MDS initial indication	Phase 3	Top-line data in 2H2019	Only HMAs approved for MDS	Worldwide issued and pending to 2037	Europe, US, Canada, Middle East, Africa
			Advanced pre-IND stage			
ON 123300	CDK4/6; ARK5	IND in 2Q2019	IND prep underway	Palbociclib	Issued US, EP	Ex-China rights

For further information please visit our website: www.onconova.com