Onconova Therapeutics (ONTX)

Onconova Reported Second Quarter 2017 Financial Results and Provided a Clinical Update

Earlier today, Onconova Therapeutics (NasdaqCM: ONTX) released financial results for the second quarter of 2017 and provided an update on its recent progress. The Company expects to report interim data from the Phase III INSPIRE trial with IV rigosertib in patients with high-risk myelodysplastic syndromes (HR-MDS) during the fourth quarter of 2017. Onconova also intends to begin a Phase III trial for oral rigosertib in combination with azacitidine in first-line HR-MDS during 2018. The Company intends to submit a Special Protocol Assessment (SPA) to the FDA for this trial. To support the oral Phase III study, Onconova has continued to enroll patients into an expansion cohort of a Phase II trial assessing the combination regimen. As of June 30th, Onconova had cash and cash equivalents of $15 million, which is expected to fund operations into 2018.

- **Interim Analysis Expected in Q4 from INSPIRE Study.** Onconova is focused on the Phase III INSPIRE study evaluating IV-rigosertib. The study is expected to enroll about 225 patients with high-risk MDS who are refractory to prior hypomethylating agents, randomized 2:1 to IV rigosertib and best supportive care (BSC), or physician’s choice of treatment plus BSC. Patients must be less than 82 years of age and cannot have received HMA therapy for more than 9 months and/or 9 cycles over 12 months. The study analysis is planned to evaluate a predefined IPSS-R very high-risk subgroup, and ITT population, which provide additional opportunities for potential positive data.

Onconova intends to perform an interim analysis following 88 deaths in this study, which is expected to occur during the fourth quarter of 2017. On the call, management mentioned that enrollment has been slower than expected, which it partly attributed to seasonality, and anticipates it will pick up. Timing on topline data should be clearer following the interim analysis.

- **Phase III Clinical Program for First-Line HR-MDS Planned to Begin in 2018.** Regarding the Company’s planned Phase III clinical trial with oral rigosertib in combination with azacitidine for first-line HR-MDS, Onconova reported that it intends to begin the Special Protocol Assessment (SPA) process after completion of the Phase II expansion study using the combination treatment in 40 patients. The planned Phase III study is expected to be a be a randomized, double-blind, placebo controlled trial evaluating the combination of oral rigosertib and azacitidine compared to azacitidine plus placebo as a first-line treatment for patients with HR-MDS. The primary endpoint is expected to be overall response rate (ORR). The use of a response-based endpoint is designed to reduce the time and cost needed to complete the trial, thereby allowing quicker data readouts. To run this study, management noted that it will need additional financing.

**Expected Upcoming Milestones**

- Q4-2017 – Interim analysis of Phase III INSPIRE trial.
- 2018 – Commencement of Phase III trial using oral rigosertib in combination with azacitidine for first-line HR-MDS.
Expansion of a Phase II Study for Rigosertib in Combination with Azacitidine May Provide Momentum for Phase III Trial. Onconova is conducting an extended portion of a Phase II study using oral rigosertib and azacitidine in 40 patients with first-line HR-MDS. The goal of this study is to determine the optimal dosing schedule for the planned Phase III study. The first patient was recruited in April, and management noted that it expects recruitment to be quicker than the study for second line patients. Overall, the expansion Phase II study could provide momentum for the global Phase III trial since the sites used in the expansion study may act as seed sites.

Prior Data Support Ongoing and Future Studies. Recall previous Phase I/II data with oral rigosertib and azacitidine in combination showed encouraging response rates as compared to azacitidine monotherapy in first and second line MDS patients. The Company reported a 76% response rate according to IGW response criteria including 8 complete remissions. Among HMA-naive patients, the response rate was 85% (17/20), as compared to 62% (8/13) for HMA-resistant patients. In the HMA-naive group 7 of 20 achieved a CR (35%), while 1 of 13 HMA-resistant patients had a CR (8%). The combined median duration of CR was 8.0 months. Although caution should be raised when comparing data across trials, these results are favorable in light of studies showing 6-17% CR and an expected response rate of roughly 45% for first-line MDS patients receiving azacitidine monotherapy.

Second Quarter 2017 Financial Results. As of June 30th, Onconova had cash and cash equivalents of $15.0 million, which is expected to fund operations into 2018. Research and development expenses for the second quarter totaled $4.6 million, as compared to $5.6 million during the second quarter of 2016. General and administrative expenses were $1.8 million for the second quarter of 2017, as compared to $2.1 million during the second quarter of 2016. The Company reported a net loss of $2.6 million, or $0.29 per share for the second quarter, as compared to $5.4 million, or $0.29 per share during the same period in 2016.

Risk to Investment

We consider an investment in Onconova Therapeutics to be a high-risk investment. Onconova Therapeutics is a clinical stage biopharmaceutical company. The Company’s lead candidate did not meet the primary endpoint in a Phase III trial, and an additional Phase III with a particular subset of patients is ongoing. This clinical trial will result in significant expenses to the Company and may require additional rounds of dilutive financing. As with any company, Onconova Therapeutics may be unable to obtain sufficient capital to fund planned development programs. There are regulatory risks associated with the development of any drug, and Onconova Therapeutics may not receive FDA approval for its candidates despite significant time and financial investments. Regulatory approval to market and sell a drug does not guarantee that the drug will penetrate the market, and sales may not meet expectations.
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