Treating Refractory Hematological Malignancies – Myelodysplastic Syndromes (MDS) and Acute Myeloid Leukemia (AML): Emerging Therapeutics

Description: Targeting unmet needs in the treatment of cancer through innovative drug development strategies have witnessed favourable outcomes recently. New targets have been validated in this process where kinases lead the way. The success has boosted the innovative biotech sector and is reflected in the performance of the IPOs’ since 2010 till date. Positive clinical data/ regulatory approval, lucrative partnership/ acquisition announcement have increased the confidence of investors on innovation and whetted their appetite for risk associated with innovation!

Several companies that have recently made their debut in public (NASDAQ) are addressing unmet needs/ niche indications with novel technology platforms/targets. Earlier, the authors had highlighted the investment opportunity and drugs being developed for Relapsed and Refractory Multiple Myeloma (RRMM). Approval of Kyprolis (carfilzomib, Onyx), Pomalyst (pomalidomide, Celgene) for this indication and their successful commercial launch validate the strategy deployed by the companies. They expect a similar scenario to unfold in the treatment of recurrent/ refractory MDS (RR-MDS, High-Risk MDS), and AML (relapsed/ refractory AML, R/R AML) and in this report highlight the new drugs and companies developing these drugs.

Innovative approach to treating unmet need has been handsomely rewarded in the past through in-licensing or outright acquisition of the mature/nascent product/innovator company at a premium. The successful IPOs’ of Ambit and Onconova are indicators of the optimism prevailing amongst the investors, Patients and Physicians community. In this report, details of some of these deals are included where acquirers like Celgene, Takeda, Astellas, Roche etc are reporting robust sales and progress in pipeline from these acquisitions.

This once again reiterates the thesis that R&D focus is essential and worth the risk for a pharma company. While some of the compounds are still in the clinic or even returned to the innovators on ground of strategic decisions taken by the management, it has not discouraged new companies from pursuing new targets and compounds leaving room for in-licensing and/or M&A opportunity drugs in the early stages of development. The report highlights some of those compounds available for partnering- “tapping them young”.

KEY POINTS DISCUSSED IN THE REPORT –

Overview of the disease and Unmet need

Drugs in the pipeline – Mechanism of Action(MoA) and Clinical Stage of development

- MDS: Lower-Risk, Higher-Risk and recurrent/refractory MDS
- AML: Untreated/ newly diagnosed and Relapsed or Refractory AML

Key milestones

Launch Timeline and Commercial Opportunity of Late-stage pipeline (RR MDS and RR AML)

M&A and Licensing deals in the last 5 years

In-licensing and/or M&A Opportunity

Detailed Company analysis: Includes Clinical data of drugs, Milestones, and Valuation

1. Ambit Biosciences (AMBI)
2. Onconova Therapeutics (ONTX)
3. Celgene (CELG)
4. SymBio (4582)
1. Executive Summary

2. Investment Drivers of The Select Companies Covered

3. Myelodysplastic syndrome (MDS)
3.1 Disease Overview
3.2 Unmet Need
3.3 Current Standard of Care
3.3.1 Supportive Care
3.3.2 Low-Intensity Therapy
3.3.3 High-Intensity Therapy
3.3.4 Recurrent/ Refractory or relapsed MDS

4. Acute Myelogenous Leukemia (AML)
4.1 Disease Overview
4.2 Current Standard of Care
4.2.1 AML pts younger than 60 years
4.2.2 AML pts Older than 60 years
4.3 Unmet Need
4.4 Refractory or relapsed AML

5. Competitive Landscape: RR/ High-Risk MDS and RR AML

6. Competitive Landscape: Untreated/ Newly Diagnosed MDS and AML

7. Key Milestones

8. Launch Timeline and Commercial Opportunity of Late-stage Pipeline (RR MDS and RR AML)

9. Drivers of M&A / Licensing Deals in MDS/ AML
9.1 Select M&A Deals in Last 5 Years – 2007 to 2012
9.2 Select Licensing Deals in Last 5 Years – 2007 to 2012
9.3 M&A and Licensing Deals Opportunity Detailed Company Analysis (incl. Clinical Data of Drugs, Milestones, and Valuation)

10.1 Ambit Biosciences (AMBI)
10.1.1 Investment Drivers
10.1.2 Other Assets – Not Fully valued in the Price
10.1.3 Key Milestones
10.1.4 NPV Valuation – Scenario Analysis (Early Approval)
10.1.5 Background about AMBI's products
   a. Quizartinib – Key profile, Clinical Data, IP protection
   b. Other assets – CEP-32496, AC410, & AC708
   c. Competitive Landscape – Late- and Mid-stage AML drugs
   d. Select JAK 2/1 Inhibitor: Late- and Mid-stage Pipeline
10.2 Onconova Therapeutics (ONTX)
10.2.1 Investment Drivers
10.2.2 Upside from Early-stage pipeline
10.2.3 Strong IP position
10.2.4 Key Milestones
10.2.5 NPV Valuation
10.2.6 Background about ONTX's products
   a. Estybon (rigosertib) – Key Profile, Primary Indications
   b. Rigosertib IV – 2nd-line Higher-Risk MDS (clinical data and competition landscape)
   c. Rigosertib Oral – 1st-line Lower-Risk MDS (clinical data and competition landscape)
   d. Estybon (rigosertib) – Label Expansions Metastatic Pancreatic cancer Head and Neck cancer
   e. Other Early-stage drugs – ON 013105, Recilsib
   f. Preclinical Programs – ON 1231320, ON 123300, ON 108600,ON 044580
   g. Patents
   h. Collaboration/ Partnership Agreements

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