

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

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## **Abstracts**

The report provides an overview of the approved therapies for AML, MDS, unmet need and limitations of the current SOC for relapsed and refractory pts. The report highlights the competition and commercial opportunity in pursuing this therapy area – Comprehensive list of the early and late stage drugs in the clinic, their MoA and the companies developing them.

Detailed financial and competitive analysis of the companies leading in the race to launch new therapies for this indication – Ambit Biosciences, Onconova Therapeutics, Symbio Pharma, and Celgene are available as a separate or with the full report. Key M&A activities that have taken place in this area in the last 5 years and a list of products in early /late stages of development available for in-licensing are highlighted in the report. This report is built using primary and secondary research data and in-house proprietary database.

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## About

Targeting unmet needs in the treatment of cancer through innovative drug development strategies have witnessed favorable 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, we had highlighted the investment opportunity and drugs being developed for Relapsed and Refractory Multiple Myeloma (RRMM). Approval of Kvpolis (carfilzomib, Onyx), Ponialyst (pomalidomide, Celgene) for this indication and their successful commercial launch validate the strategy deployed by the companies. We 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".

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