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)
Contents:

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
         a. Induction Therapy
      4.2.2 AML pts Older than 60 years
         a. Current treatment options – Hypomethylating agents and SCT
   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. 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. Quizzartinib – 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, Recilisib
       f. Preclinical Programs – ON 1231320, ON 123300, ON 108600, ON 044580
       g. Patents
       h. Collaboration/ Partnership Agreements

Order by Fax - using the form below

Order by Post - print the order form below and send to

Research and Markets,
Guinness Centre,
Taylors Lane,
Dublin 8,
Ireland.
Fax Order Form
To place an order via fax simply print this form, fill in the information below and fax the completed form to 646-607-1907 (from USA) or +353-1-481-1716 (from Rest of World). If you have any questions please visit
http://www.researchandmarkets.com/contact/

Order Information
Please verify that the product information is correct and select the format(s) you require.

Product Name: Treating Refractory Hematological Malignancies – Myelodysplastic Syndromes (MDS) and Acute Myeloid Leukemia (AML): Emerging Therapeutics
Web Address: http://www.researchandmarkets.com/reports/2635096/
Office Code: SCBR9VGZ

Product Formats
Please select the product formats and quantity you require:

<table>
<thead>
<tr>
<th>Format</th>
<th>Quantity</th>
<th>Price</th>
</tr>
</thead>
<tbody>
<tr>
<td>Hard Copy</td>
<td></td>
<td>USD 2500 + USD 58 Shipping/Handling</td>
</tr>
<tr>
<td>CD-ROM</td>
<td></td>
<td>USD 2500 + USD 58 Shipping/Handling</td>
</tr>
<tr>
<td>Electronic (PDF) - Single User</td>
<td></td>
<td>USD 2000</td>
</tr>
<tr>
<td>Electronic (PDF) - Site License</td>
<td></td>
<td>USD 5000</td>
</tr>
<tr>
<td>Electronic (PDF) - EnterpriseWide</td>
<td></td>
<td>USD 7500</td>
</tr>
</tbody>
</table>

* Shipping/Handling is only charged once per order.

Contact Information
Please enter all the information below in BLOCK CAPITALS

Title: Mr □ Mrs □ Dr □ Miss □ Ms □ Prof □
First Name: __________________________ Last Name: __________________________
Email Address: * __________________________
Job Title: __________________________
Organisation: __________________________
Address: __________________________
City: __________________________
Postal / Zip Code: __________________________
Country: __________________________
Phone Number: __________________________
Fax Number: __________________________
Payment Information

Please indicate the payment method you would like to use by selecting the appropriate box.

☑ Pay by credit card: You will receive an email with a link to a secure webpage to enter your credit card details.

☐ Pay by check: Please post the check, accompanied by this form, to:
Research and Markets,
Guinness Center,
Taylors Lane,
Dublin 8,
Ireland.

☐ Pay by wire transfer: Please transfer funds to:
Account number 833 130 83
Sort code 98-53-30
Swift code ULSBIE2D
IBAN number IE78ULSB98533083313083
Bank Address Ulster Bank,
27-35 Main Street,
Blackrock,
Co. Dublin,
Ireland.

If you have a Marketing Code please enter it below:

Marketing Code: ____________________________

Please note that by ordering from Research and Markets you are agreeing to our Terms and Conditions at http://www.researchandmarkets.com/info/terms.asp

Please fax this form to:
(646) 607-1907 or (646) 964-6609 - From USA
+353-1-481-1716 or +353-1-653-1571 - From Rest of World