Mirum Pharmaceuticals (NASDAQ:MIRM)

July 10, 2020

CATALYST SUMMARY

Progressive familial intrahepatic cholestasis (PFIC) is rare liver disease in children that begins in infancy and involves an interruption in the transfer of bile acid to the liver. There are three subtypes, PFIC1, PFIC2 and PFIC3, and each has a distinct genetic signature. Children with PFIC1 can experience intense itching, poor weight gain and stunted growth. PFIC2 typically relates only to liver disease. More seriously, all forms of PFIC can lead to cirrhosis and liver failure within the first 10 years of life.

No standard medical therapy other than surgery has proven effective in treating PFIC. Mirum Pharmaceuticals is developing maralixibat to treat PFIC2. *SanaCurrents* **assigns a high pivotal sentiment** the company will report positive, peer-reviewed data from its maralixibat phase II trial in PFIC at the Digital International Liver Congress 2020 between August 27-29, 2020.

PFIC afflicts between 1 in every 50,000 to 100,000 children around the world. Physicians have reported some success in treating PFIC with ursodeoxycholic acid. But the results are sporadic, and the drug usually is discontinued after an initial attempt at treatment. As the disease progresses, surgical options include a procedure known as partial external biliary diversion and liver transplantation. Both carry substantial risk for young children.

In a recent study conducted by a global consortium of pediatric hepatologists, the authors concluded serum bile acid (sBA) control (<102µmol/L or a 75% reduction) after biliary diversion surgery was associated with native liver survival of up to 15 years. Similarly, results from an earlier maralixibat trial indicated the drug surpassed the sBA thresholds described by the consortium. The data from the maralixibat responders were **statistically significant compared to baseline (p<0.05).** In addition, maralixibat responders achieved normalization of liver enzyme and bilirubin levels, decreased pruritus, and improved z-scores in both height and weight.

The Digital International Liver Congress selected the consortium's maralixibat findings for peer review. Mirum will present the phase II data at the congress in late August to include responses over the last three months.

THE EDGE

Mirum's stock climbed 25% in June on previous news of the phase II data, prior to peer review. While drug companies and academic researchers frequently can find publications to publish their data, the International Liver Congress is recognized for its rigor. Publication of the maralixibat data in late August will validate the drug's efficacy further.

Maralixibat and Mirum's second drug, volixibat, previously were validated by a private company and Shire International, via investment. The two drugs

originally were developed by privately held Lumena Pharmaceuticals. Shire then paid more than $260 million up front to acquire Lumena in 2014. Before 2018, Shire acquired Baxalta in a $31 billion deal and then found itself a target of a $62 billion takeout by Takeda (TSE:4502/NYSE:TAK), completed in January 2019.

PROBABILITY SENTIMENT

- ☐ SUPERIOR (highest)
- ☑ Pivotal
- ☐ Advantageous

Key Catalyst(s)

- Maralixbat phase II data in PFIC2
- Rolling NDA submission

Key Catalyst Date(s)

- August 27-29, 2020, phase II data in PFIC2
- September 30, 2020, NDA

Insider & Institutional Holdings

10.32% % of Shares Held by All Insider

73.60% % of Shares Held by Institutions

82.07% % of Float Held by Institutions

67 Number of Institutions Holding Shares

Key Executives

Mr. Christopher Peetz, Pres, CEO & Director

Dr. Pamela Vig Ph.D., Chief Scientific Officer

Ms. Lara Longpre MSC, MBA, Chief Devel. Officer

Dr. Ian Clements, Chief Financial Officer

Mr. Peter Radovich, Chief Operating Officer

Location

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General Guidelines for Probability Sentiments

Based on SanaCurrents' analytical model, Superior and Pivotal sentiments reflect a probability score of at least 60% that the company will announce a positive result to the specified catalyst. In our experience, a positive result typically increases the company's share price. An Advantageous sentiment reflects a score of less than 60%.

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THE EDGE cont'd

As a result, Shire licensed maralixibat and volixibat for $120 million to Mirum in November 2018. The management at Mirum includes three former Lumena executives, including CEO Chris Peetz, who continued to advance the two drugs.

Mirum already has moved maralixibat into a phase III trial in PFIC. The phase III trial is designed to treat 30 patients for 26 weeks with maralixibat or a placebo.

The company also is testing maralixibat in another rare pediatric liver disease, Alagille syndrome (AS). Maralixibat has received Breakthrough Therapy Designation for both PFIC and Alagille syndrome. Mirum expects to begin a rolling NDA submission for maralixibat in AS before September 30.

Shares of Mirum shot up to December 2019 to $25.90 per share from $8.19 per share, on news the FDA would be willing to review the rolling NDA submission in AS.

Because maralixibat has earned a rare pediatric disease designation in AS, Mirum is eligible to receive a priority review voucher if the drug gains approval. Priority review vouchers are transferable, meaning Mirum can sell the voucher to a larger drug company if it chooses. Sales of priority review vouchers, which can reduce FDA review time by four months of a key drug a large pharma wants to push to the market, can yield $300 million or more to a seller such as Mirum. In effect, the voucher can become non-dilutive cash to the seller, partly explaining the spike in shares in December 2019.

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Tear Sheet

Mirum Pharmaceuticals, Inc. USD 19.03 -0.64 -3.25%

Country	United States of America	Next FQ/FH End Date	Jun 30, 2020
Exchange	NASDAQ Stock Market	Next FY End Date	Dec 31, 2020
GICS Sector	Health Care	Next earnings Date	Aug 06, 2020
GICS Industry	Biotechnology	Latest earnings Date	May 07, 2020
Currency	USD		

General information **in millions USD, except per share data*

Price (Previous Close)	19.03	Div. Yield, Last year (FY0)	
Price - 52 Week High	29.31	Shares Outstanding	22.6 MM
Price - Current vs 52wk High	-32.78%	Short Interest Shares	0.5 MM
Price - 52 Week Low	6.81	SI % of O/S	2.41%
Price - Current vs 52wk Low	192.30%	Market Capitalization	403
Beta - Sampling last 1y, Daily	vs. S&P 500	Net Debt	-117
		Enterprise Value	334

Estimates revision momentum **in millions USD, except EPS*

	Last reported FY	Current Year FY1			Next Year FY2		
		6mth ago	3mth ago	Today	6mth ago	3mth ago	Today
Consensus date		1/11/2020	4/11/2020	7/11/2020	1/11/2020	4/11/2020	7/11/2020
Revenue	0	0	0	0	0	15	14
% diff to today		n/a	n/a		n/a	-100.0%	
Gross Profit	0					15	14
% diff to today		n/a	n/a		n/a	-4.9%	
EBIT	-55	-51	-65	-63	-77	-114	-131
% diff to today		60.2%	0.8%		70.0%	15.0%	
EBITDA	-55						
% diff to today		n/a	n/a		n/a	n/a	
EPS	-4.58	-3.86	-3.44	-3.66	-3.41	-4.16	-4.57
% diff to today		-7.0%	-1.3%		-10.0%	-2.2%	

Consolidated Income Statement **in millions USD, except diluted EPS (adj.) and common shares*

	FY-5	FY-4	FY-3	FY-2	FY-1	FY0	3yr-CAGR
Period end date	12/31/2014	12/31/2015	12/31/2016	12/31/2017	12/31/2018	12/31/2019	
Sales					0	0	
Gross Income					0	0	
EBITDA (adj.)					-3	-55	
EBIT (adj.)					-3	-55	
Earnings Before Tax (adj.)					-3	-63	
Net Income (adj.)					-3	-63	
Diluted EPS (adj.)					-0.12	-4.58	
Common Shares					23	23	-1.0%

Margins & Profitability Ratios

	FY-5	FY-4	FY-3	FY-2	FY-1	FY0	vs. last 5y average (%pts)
Period end date	12/31/2014	12/31/2015	12/31/2016	12/31/2017	12/31/2018	12/31/2019	
Gross Margin							
R&D % of Sales							
SG&A % of Sales							
EBITDA Margin (adj.)							
EBIT Margin (adj.)							
Net Margin							
Tax Rate					0.0%	0.0%	
ROA						-52.9%	
ROE						-59.4%	

Per Share Metrics **in USD*

	FY-2	FY-1	FY0	FY1
Book Value:		-0.45	5.77	
Free Cash Flow:		-0.34	-1.75	
Dividend:		0.00		0.00
Earnings (EPS):		-0.12	-4.58	-3.56

Valuation ratios

	LTM	NTM	FY1	FY2
EV/Sales				22.7 x
EV/EBITDA	nm			
P/E	nm	nm	nm	nm
PE to Growth (PEG)				
EV/FCF	nm			
P/Sales				33.9 x
P/BV	5.5 x			
P/Tangible BV	5.5 x			

Consolidated Balance Sheet **in millions USD*

	FY-2	FY-1	FY0
Period end date	12/31/2017	12/31/2018	12/31/2019
Cash & ST Investments		52	117
Inventory			
Total Assets		52	147
Current Liabilities		2	12
Total Debt		0	0
Total Liabilities		2	16
Total Shareholders Equity		50	130

Consolidated Cash Flow Statement **in millions USD*

	FY-2	FY-1	FY0
Period end date	12/31/2017	12/31/2018	12/31/2019
Net Income		-3	-63
D&A		0	0
Change in WC		2	7
Cash From Operations		-1	-35
Capital Expenditures		-0	0
Cash From Investments		-0	-128
Shares Repurchased			
Cash From Financing		69	127
Cash & Cash Eq - Period end		52	12

Disclosure of General Guidelines for SanaCurrents Strategy

In most situations, SanaCurrents partners take a position in a stock (or buy shares) shortly after a report on a catalyst is published. This is particularly true if the catalyst is within 90 days of the published report. For catalysts with horizons of 6-9 months, or longer, SanaCurrents anticipates the biopharma and device stocks from which it selects catalysts can rise or fall 20-30% in the months following the date of the published report. While buying at a dip, naturally, is preferred, SanaCurrents does not forecast or model in swings in share price prior to the catalyst. SanaCurrents' analytics score models the probability a compelling catalyst will be positive. SanaCurrents expects a positive announcement regarding a catalyst will drive a company's stock to higher level than the price on the date of the report, based on the company's value and capital structure when the report was published. That said, subscribers should exercise their preferred, individual discipline when timing trades, buy or sell, regarding the catalyst forecast.

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