LAIDLAW & COMPANY
Est. 1842
London
New York
San Francisco
Stamford
Ft. Lauderdale

EQUITY RESEARCH
Company Report

April 9, 2015

Mast Therapeutics (MSTX - $ 0.50)

Healthcare/Biotechnology

Vepoloxamer Pre-clinical Data to Be Presented at Sickle Cell Disease Annual Meetings

Ticker:	**MSTX**
Rating:	**Buy**
Price Target:	**$ 2.50**

This morning, MSTX announced it will report vepoloxamer pre-clinical study results, and will also present at both the 9th Annual Sickle Cell Disease Research & Educational Symposium and the 38th National Sickle Cell Disease Scientific Meeting (to be held April 10-13, 2015).

- **Details.** MSTX announced this morning the upcoming presentation of vepoloxamer pre-clinical study results from a poster entitled "Evaluation of purified poloxamer-188 (vepoloxamer) on sickle red blood cell (RBC) adhesion and membrane fragility utilizing microfluidic-based flow adhesion bioassays" at the 9th Annual Sickle Cell Disease Research & Educational Symposium. The study illustrated that based on a microfluidics-based assessment of blood samples from 12 sickle cell patients, vepoloxamer exhibited dose-dependent and statistically significant reductions in the adhesive properties and fragility of red blood cells. During the aforementioned presentations, MSTX management will discuss the potential of vepoloxamer as a treatment in resolving vaso-occlusive crisis (VOC) in SCD patients, and additional communication devices developed by MSTX to facilitate the access by SCD patients.
- **Implications.** Although the major focus of investors remains on the progression of the Phase III (EPIC) study, we believe the presentations at the SCD medical conferences could afford more awareness and familiarity about vepoloxamer to physicians and patient advocate groups. Awareness is important, given that the potential top-line results could be three quarters away (study completion expected in 4Q15 with top-line results available in 1Q16). The pre-clinical study results are consistent with the clinical effects vepoloxamer demonstrated during the prior studies, in our view. Although Pfizer recently announced that the company plans on initiating a Rivipansel in resolving VOC of SCD patients Phase III clinical study in mid-2015; we believe MSTX's vepoloxamer development remains very much ahead of the competition and with a substantial lead time.
- **Action.** We are reiterating our Buy rating and our $2.50 target price based on peer comparable probability adjusted DCF analyses to reflect the continued execution of corporate developments. We view the MSTX story as under-exposed and the shares as under-valued, in our opinion.

Trading Data:

Last Price (04/09/2015)	$ 0.50
52–Week High (6/24/2014)	$ 0.73
52–Week Low (11/6/2014)	$ 0.40
Market Cap. (MM)	$ 80
Shares Out. (MM)	159

Earnings Estimates: (per share)

(Dec)	1Q	2Q	3Q	4Q	FY	P/E
FY-15E	-0.05	-0.06	-0.06	-0.06	-0.24	N.A.
FY-14A	-0.06	-0.06	-0.06	-0.05	-0.23	N.A
FY-13A	-0.12	-0.09	-0.05	-0.06	-0.28	N.A.
FY-12A	-0.09	NA	-0.07	-0.08	-0.33	N.A

Source: Laidlaw & Company estimates

Yale Jen, Ph.D.
Managing Director/Senior
Biotechnology Analyst
(212) 953-4978
yjen@laidlawltd.com

Abstract: Evaluation of purified poloxamer-188 (vepoloxamer) on sickle red blood cell (RBC) adhesion and membrane fragility utilizing microfluidic-based flow adhesion bioassays

Patrick C. Hines[1], Jennell White[1], Xiufeng Gao1, Ke Liu[1], Marty Emanuele[2]; [1]Functional Fluidics, Detroit MI, [2]Mast therapeutics, Inc. San Diego, CA.

Background Sickle erythrocyte adhesion and membrane fragility contribute to vaso-occlusion and downstream tissue and organ ischemia. Vepoloxamer is an amphipathic triblock copolymer with multi-mechanistic properties believed to improve these pathophysiologic consequences, by sealing damaged cell membranes, inhibiting hydrophobic cellular interactions, and facilitating thrombolysis. Studies have also shown the reduction in both acute vaso-occlusive crisis duration and total opioid analgesic requirements. A phase 3 clinical trial of the effectiveness of vepoloxamer in attenuating acute vaso-occlusive events is currently underway. Because of the significant patient to patient variability in sickle cell disease (SCD) vaso-occlusive phenotype, we evaluated the effects of vepoloxamer on sickle blood to identify individual patients at greater risk of pathologic vascular adhesion, predict if and to what extent vepoloxamer may improve these pathologies, and thus guide its therapeutic use.

Objective This study was to evaluate the effects of vepoloxamer on whole blood adhesive properties and RBC membrane fragility of individual SCD patients.

Methods Blood was obtained from pediatric homozygous sickle cell patients (n = 12) at steady state. The dose response to vepoloxamer was evaluated by measuring whole blood adhesion to vascular cell adhesion molecule (VCAM-1) during physiologic flow conditions. Erythrocyte membrane fragility was measured based on mechanical stress-induced hemolysis at 3 min (Hem_{3min}, %) and 10 min (Hem_{10min}, %).

Results Compared to control, blood samples treated with vepoloxamer (n=12) at 0.1 mg/mL demonstrated an 18% decline of the median number of adherent cells ($p = 0.0015$). At 1.0 mg/mL and 10.0 mg/mL, a 69% and 79% reduction in adherent cells was observed respectively (in both cases, $p < 0.001$). Vepoloxamer also reversed established cell adhesion, and the degree of adhesion reversal varied from patient to patient. In addition, vepoloxamer reduced shear-induced hemolysis compared to untreated blood samples (n = 10). Although these reductions varied patient to patient (e.g. 2.2 to 49.4% for Hem_{3min}), statistical significance was reached at 3 min application of shear stress (Hem_{3min}) ($p = 0.033$).

Conclusion Vepoloxamer reduces whole blood adhesive properties in a dose-dependent manner, and reduces shear-induced hemolysis in a statistically significant manner. This pre-clinical approach to assessing the response of adhesive properties and membrane fragility to vepoloxamer treatment may facilitate selection of patients most likely to benefit from vepoloxamer therapy. Additionally, this approach may provide a mechanism to monitor an individual patient's response to vepoloxamer over time both in clinical studies and in patient therapy.

Source: 9th Annual Sickle Cell Disease Research and Educational Symposium

Anticipated milestones in 2015 and beyond

Product	Indication	Event	Timing	Importance
MST-188	Vaso-occlusive crisis (VOC) in sickle cell disease (SCD)	Periodical updates on Phase III trial progress	**2014/2015**	***
		Start EPIC extension (repeat exposure):study	**1H15**	***
		Completion of Phase III (EPIC) study	**4Q15**	***
		Report of Phase III study top-line results	**1Q16**	****
		Potential NDA filing	**2H16**	***
		Potential approval	**2017**	****
	Acute limb ischemia	Report Phase II study top-line results	**2H16**	***
	Chronic heart failure	Potentially report Phase II top-line results	**2016**	***
AIR001	PH associated with heart failure with preserved ejection fraction (HFpEF),	Potentially start investigator-sponsed Phase II study	**2015**	***
		Report preliminary results	**2H15**	****

***** / ***** Major catalyst event that could impact share price very significantly while *** event is more informative*

Source: Laidlaw & Company and company presentation

Yale Jen, Ph.D.
yjen@laidlawltd.com

Major Risks

Risks of clinical study failure could have a major impact on MSTX share value. Despite an encouraging prior Phase III study outcome and potentially favorable trial design of the ongoing EPIC Phase III study, risks still exist that MST-188 might not receive approval by the FDA if the pivotal study does not meet its endpoints. Given that the majority of upside for MSTX shares is currently based on the assumption that a positive EPIC study outcome could lead to MST-188 approval before its commercial potential can be realized; a failure of the EPIC study would have a significant negative impact on MSTX share value.

Commercial success of the MST-188 in resolving vaso-occlusive crisis in sickle cell disease (SCD) patients is less predictable. Although we recognize that the substantial unmet medical need of shortening the VOC in SCD patients, we cannot fully predict the market acceptance and potential revenue ramp up for the product; as the actual clinical performance will play an important role even if the product is approved. In addition, we cannot fully foresee the market dynamic if competitors also enter the market since such dynamic could be affected by multiple factors. Together, commercial performance of MST-188 may not meet expectations, and if so, MSTX share value could also be impacted negatively.

Lack of patent protection could make MST-188 vulnerable if competitors develop method to generate a me-too product that might not require complete clinical studies but as generic. Although the production processes of MST-188 are protected on several fronts, including proprietary technology, trade secrets and know-how, it remains possible that other competitors could develop similar or alternative processes to produce a similar, or even better, product like MST-188. As such, the company might not enjoy the competitive edge and potentially damage MST-188's commercial outlook.

Limited product diversity could increase overall risk. Given the nascent stage of the corporate development, majority of the product pipeline value mainly resides on MST-188 in SCD development. The second potential pipeline product, AIR001 in pulmonary arterial hypertension and additional indications (acute limb ischemia, stroke and acute heart failure), potentially could be addressed by MST-188 remains in very early development stage. As such, we believe the company at the current stage has very limited diversification potential in its product pipeline.

Additional financing could dilute shareholder value. Although the company ended 3Q14 with ~$43MM cash, MSTX could potentially need more financial resources going forward if they want to expand and further develop its pipeline and/or commercialize MST-188 in SCD. Should the product not receive FDA approval or product revenue does not reach expectations, the company might need to increase its financial resources, which includes issuing new equity to raise additional cash. Under such a scenario, the share value of existing shareholders could be diluted.

Yale Jen, Ph.D.
yjen@laidlawltd.com

Figure 1: Income Statement

Mast Therapeutics – Income Statement

($'000)	2011	2012	2013	2014	1Q15E	2Q15E	3Q15E	4Q15E	2015E	2016E	2017E	2018E	2019E	2020E
Revenue														
MST-188 revenue	0	0	0	0					0	0	28,253	86,796	167,886	252,822
Net sales	0	0	0	0	-	-	-	-	0	0	0	0	0	0
Licensing revenue	0	0	0	0	-	-	-	-	0	0	0	0	0	0
Grant revenue	0	0	0	0	-	-	-	-	0	0	0	0	0	0
Total revenue	0	0	0	0	-	-	-	-	0	0	28,253	86,796	167,886	252,822
Costs of goods	0	0									2,543	7,812	15,110	22,754
Research and development	5,758	8,088	12,902	19,436	5,525	6,022	6,504	6,894	24,945	26,692	22,688	21,553	22,200	22,866
Selling, general and administrative	7,190	7,519	8,518	9,487	2,451	2,490	2,535	2,573	10,050	10,351	11,283	12,298	13,405	14,478
Marketing and sales											20,000	23,000	24,380	25,599
Transaction-related expenses	411	(70)	80	271	0	-	-	-	0	0	0	0	0	0
Depreciation and amortization	38	90	40	84	25	25	25	25	100	100	100	100	100	100
Total Operating Expenses	13,397	15,628	21,539	29,279	8,001	8,538	9,064	9,492	35,095	37,143	56,613	64,763	75,195	85,797
Operating Incomes (losses)	(13,397)	(15,628)	(21,539)	(29,279)	(8,001)	(8,538)	(9,064)	(9,492)	(35,095)	(37,143)	(28,360)	22,032	92,691	167,025
Reduction of fair value of warrants	0	0	0	0	0	-	-	-	0	0	0	0	0	0
Investment income	66	74	60	67	17	17	17	17	68	75	82	91	100	110
Interest expense	0	0	0	0	0	-	-	-	0	0	0	0	0	0
Other income/(expense), net	71	(5)	(1)	511	50	50	50	50	200	2	(20)	24	(27)	(27)
Loss before cumulative effect of change in accounting princ	(13,260)	(15,559)	(21,480)	(28,701)	(7,934)	(8,471)	(8,997)	(9,425)	(34,827)	(37,066)	(28,298)	22,147	92,763	167,108
Cumulative effect of change in accounting principle	0	0	0	0	0	-	-	-	0					
Income before tax	(13,260)	(15,559)	(21,480)	(28,701)	(7,934)	(8,471)	(8,997)	(9,425)	(34,827)	(37,066)	(28,298)	22,147	92,763	167,108
Tax	0.0	0	0	0	0	-	-	-	0	0	0	(8,194)	(34,322)	(61,830)
Net Income (Loss)	(13,260)	(15,559)	(21,480)	(28,701)	(7,934)	(8,471)	(8,997)	(9,425)	(34,827)	(37,066)	(28,298)	13,952	58,441	105,278
Net Income (Loss) Applicable to Common Shareholders	(13,260)	(15,559)	(21,480)	(28,701)	(7,934)	(8,471)	(8,997)	(9,425)	(34,827)	(37,066)	(28,298)	13,952	58,441	105,278
Net Earnings (Losses) Per Share—Basic and Diluted	($0.47)	($0.33)	($0.28)	($0.23)	($0.05)	($0.06)	($0.06)	($0.06)	($0.24)	($0.24)	($0.18)	$0.09	$0.37	$0.66
Shares outstanding—basic	28,175	47,641	76,586	122,409	146,257	147,257	148,257	149,257	147,757	154,757	155,757	156,757	157,757	158,757
Shares outstanding—diluted	28,175	47,641	76,586	122,409	146,257	147,257	148,257	149,257	147,757	154,757	155,757	156,757	157,757	158,757
Margin Analysis (% of Sales/Revenue)														
Costs of goods										9%	9%	9%	9%	9%
R&D	NA	NA	NA	NA	NA	NA	NA	NA	NA	NA	80%	25%	13%	9%
MG&A	NA	NA	NA	NA	NA	NA	NA	NA	NA	NA	111%	41%	23%	16%
Operating Income (loss)	NA	NA	NA	NA	NA	NA	NA	NA	NA	NA	-100%	25%	55%	66%
Pretax	NA	NA	NA	NA	NA	NA	NA	NA	NA	NA	-100%	26%	55%	66%
Tax Rate											37%	37%	37%	37%
Net Income	NA	NA	NA	NA	NA	NA	NA	NA	NA	NA	-100%	16%	35%	42%
Financial Indicator Growth Analysis (YoY%)														
Licensing revenue									0%	0%	0%	0%	0%	0%
Grant revenue									0%	0%	0%	0%	0%	0%
Total Revenue		NA	NA	NA	NA	NA	NA	NA	NA	NA	NA	207%	93%	51%
R&D		40%	60%	51%	29%	25%	20%	40%	28%	7%	-15%	-5%	3%	3%
SG&A		5%	13%	11%	8%	5%	3%	7%	6%	3%	9%	9%	9%	8%
Marketing and sales												15%	6%	5%
Operating Income (Losses)		17%	38%	36%	17%	19%	15%	29%	20%	6%	-24%	-178%	321%	80%
Net Income		17%	38%	34%	25%	18%	14%	29%	21%	6%	-24%	-149%	319%	80%
EPS		-31%	-14%	-16%	-11%	-7%	-5%	25%	1%	2%	-24%	-149%	316%	79%

Yale Jen, Ph.D. 212-953-4978

Source: Bloomberg LP; Company reports; Laidlaw & Company estimates

Yale Jen, Ph.D.
yjen@laidlawltd.com

DISCLOSURES:

ANALYST CERTIFICATION

The analyst responsible for the content of this report hereby certifies that the views expressed regarding the company or companies and their securities accurately represent his personal views and that no direct or indirect compensation is to be received by the analyst for any specific recommendation or views contained in this report. Neither the author of this report nor any member of his immediate family or household maintains a position in the securities mentioned in this report.

EQUITY DISCLOSURES

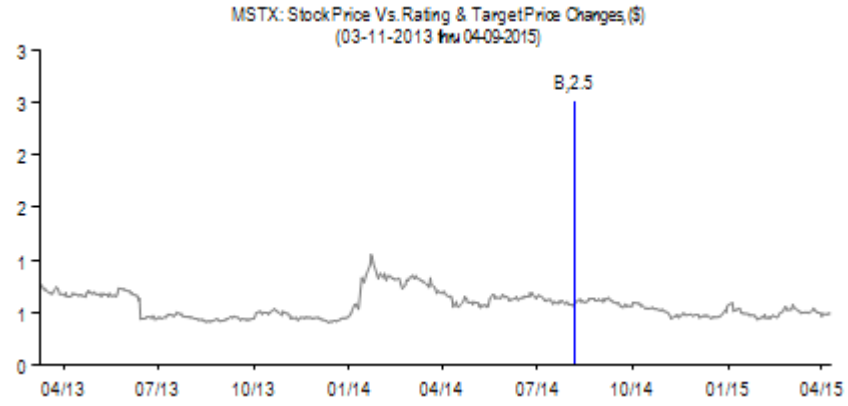
For the purpose of ratings distributions, regulatory rules require the firm to assign ratings to one of three rating categories (i.e. Strong Buy/Buy-Overweight, Hold, or Underweight/Sell) regardless of a firm's own rating categories. Although the firm's ratings of Buy/Overweight, Hold, or Underweight/Sell most closely correspond to Buy, Hold and Sell, respectively, the meanings are not the same because our ratings are determined on a relative basis against the analyst sector universe of stocks. An analyst's coverage sector is comprised of companies that are engaged in similar business or share similar operating characteristics as the subject company. The analysis sector universe is a sub-sector to the analyst's coverage sector, and is compiled to assist the analyst in determining relative valuations of subject companies. The composition of an analyst's sector universe is subject to change over time as various factors, including changing market conditions occur. Accordingly, the rating assigned to a particular stock represents solely the analyst's view of how that stock will perform over the next 12-months relative to the analyst's sector universe.

Additional information available upon request.

Laidlaw & Co (UK) Ltd. has not provided any investment banking services for the company (ies) mentioned in this report over the last 12 months.

RATINGS INFORMATION

Rating and Price Target Change History



3 Year Rating Change History

Date	Rating	Closing Price ($)
08/06/2014	Buy (B)	0.60

3 Year Price Change History

Date	Target Price ($)	Closing Price, ($)
08/06/2014	2.50	0.60

Source: Laidlaw & Company **Created by: Blue-Compass.net**

Laidlaw & Company Rating System*		% of Companies Under Coverage With This Rating	% of Companies for which Laidlaw & Company has performed services for in the last 12 months	
			Investment Banking	Brokerage
Strong Buy (SB)	Expected to significantly outperform the sector over 12 months.	0.00%	0.00%	0.00%
Buy (B)	Expected to outperform the sector average over 12 months.	68.18%	31.82%	9.09%
Hold (H)	Expected returns to be in line with the sector average over 12 months.	4.55%	0.00%	0.00%
Sell (S)	Returns expected to significantly underperform the sector average over 12 months.	0.00%	0.00%	0.00%

ADDITIONAL COMPANIES MENTIONED

GlycoMimetics (GLCY: Not Rated)
Pfizer (PFE: Not: Rated)

ADDITIONAL DISCLOSURES

As of the date of this report, neither the author of this report nor any member of his immediate family or household maintains an ownership position in the securities of the company (ies) mentioned in this report.

This report does not provide individually tailored investment advice and has been prepared without regard to the individual financial circumstances and objectives of persons who receive it. Laidlaw & Co (UK), Ltd. recommends that investors independently evaluate particular investments and strategies, and encourages investors to seek the advice of a financial adviser. The appropriateness of a particular investment or strategy will depend on an investor's individual circumstances and objectives. The securities, instruments, or strategies discussed in this report may not be suitable for all investors, and certain investors may not be eligible to purchase or participate

in some or all of them. This report is not an offer to buy or sell or the solicitation of an offer to buy or sell any security/instrument or to participate in any particular trading strategy.

Associated persons of Laidlaw & Co (UK), Ltd not involved in the preparation of this report may have investments in securities/instruments or derivatives of securities/instruments of companies mentioned herein and may trade them in ways different from those discussed in this report. While Laidlaw & Co (UK), Ltd., prohibits analysts from receiving any compensation. Bonus or incentive based on specific recommendations for, or view of, a particular company, investors should be aware that any or all of the foregoing, among other things, may give rise to real or potential conflicts of interest.

With the exception of information regarding Laidlaw & Co (UK), Ltd. this report is based on public information. Laidlaw & Co (UK), Ltd makes every effort to use reliable, comprehensive information, but we make no representation that it is accurate or complete and it should not be relied upon as such. Any opinions expressed are subject to change and Laidlaw & Co (UK), Ltd disclaims any obligation to advise you of changes in opinions or information or any discontinuation of coverage of a subject company. Facts and views presented in this report have not been reviewed by, and may not reflect information known to, professionals in other Laidlaw & Co (UK), Ltd business areas. Laidlaw & Co (UK), Ltd associated persons conduct site visits from time to time but are prohibited from accepting payment or reimbursement by the company of travel expenses for such visits. The value of and income from your investments may vary because of changes in interest rates, foreign exchange rates, default rates, prepayment rates, securities/instruments prices. market indexes, operational or financial conditions of companies or other factors. There may be time limitations on the exercise of options or other rights in securities/instruments transactions. Past performance is not necessarily a guide to future performance. Estimates of future performance are based on assumptions that may not be realized. If provided, and unless otherwise stated, the closing price on the cover page is that of the primary exchange for the subject company's securities/instruments.

The information and opinions in this report were prepared by Laidlaw & Co (UK), Ltd. For important disclosures, please see Laidlaw & Co (UK), Ltd.'s disclosure website at www.Laidlawltd.com, or contact your investment representative or Laidlaw & Co (UK), Ltd at 546 Fifth Ave, 5th Floor, New York, NY 10036 USA.

Yale Jen, Ph.D.
yjen@laidlawltd.com

NOTES:

Yale Jen, Ph.D.
yjen@laidlawltd.com