

# Portola Pharmaceuticals, Inc.

(PTLA)

SMALL & MID CAP RESEARCH



Rating **OUTPERFORM\* [V]**  
Price (05 Nov 13, US\$) 22.32  
Target price (US\$) 32.00<sup>1</sup>  
52-week price range 29.01 - 15.15  
Market cap. (US\$ m) 884.53  
Enterprise value (US\$ m) 594.40

\*Stock ratings are relative to the coverage universe in each analyst's or each team's respective sector.  
<sup>1</sup>Target price is for 12 months.

[V] = Stock considered volatile (see Disclosure Appendix).

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## Q3 Earnings: Programs On-Track; Multiple Clinical Readouts in 2014/2015

PLTA confirmed the timing of several key value inflection points in 2014/2015. Betrixaban APEX enrollment remains on track for data in mid-2015. For the Antidote program, Phase III and IV (outcomes) trials for accelerated approval will be initiated in 2014 (H1:14 for Phase III). We continue to recommend PTLA based on its diversified portfolio, retained rights, and the large addressable markets.

- **PTLA reported Q3 net loss of (\$18.6M) on collaboration revenue of \$2.8M:** PTLA expects expenses will continue to rise with BLA filing and manufacturing expense ramp.
- **Reiterate Outperform rating and a \$32 price target:** Our positive view is based on the large market opportunity for factor Xa inhibitors for clot prevention in acute medically ill patients and for its factor Xa inhibitor antidote for bleeding or surgery.
- **Well-capitalized through multiple readouts:** The company had \$218.9M in cash at the end of Q3; this does not include the recent \$100M raised in the follow-on offering. The company believes it well capitalized through Phase III read-outs for Betrixaban (inhibitor) in 2015, two or more Phase II readouts for Andexanet alfa (antidote) in 2014, and Phase I proof of concept data for PRT2070 in NHL and CLL (expected in 2014).

## Financial and valuation metrics

Year	12/12A	12/13E	12/14E	12/15E
EPS (CS adj.) (US\$)	0.44	-3.20	-3.01	-0.98
Prev. EPS (US\$)	—	-3.41	-2.99	—
P/E (x)	51.2	-7.0	-7.4	-22.7
P/E rel. (%)	306.3	-44.3	-52.2	-176.2
Revenue (US\$ m)	72.0	8.5	—	65.0
EBITDA (US\$ m)	12.2	-88.8	-120.3	-43.9
OCFPS (US\$)	-1.89	-0.14	-2.66	-0.87
P/OCF (x)	—	-158.4	-8.4	-25.7
EV/EBITDA (current)	54.6	-7.5	-5.6	-15.2
Net debt (US\$ m)	-54	-290	-172	-276
ROIC (%)	14.92	382.33	549.25	239.57
Number of shares (m)	39.63	IC (current, US\$ m)		72.78
BV/share (Next Qtr., US\$)	—	EV/IC (x)		-25.2
Net debt (Next Qtr., US\$ m)	-290.1	Dividend (current, US\$)		—
Net debt/tot cap (Next Qtr., %)	-108.9	Dividend yield (%)		—

Source: Company data, Credit Suisse estimates

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## Q3 Ahead of Our Forecast

PTLA reported Q3 net loss of (\$18.6M) on collaboration revenue of \$2.8M. This is better than our forecast net loss of (\$24.5M) on revenue of \$1.8M. Our full-year 2013 and 2014 EPS estimates slightly change due to the upside in Q3.

## Antidote Timelines on Track

Andexanet alfa (aka PRT4445) is a recombinant biologic designed to reverse the anticoagulant activity of oral and injectable factor Xa inhibitors in patients who suffer serious bleeds or require surgery while on these therapies.

### PTLA confirmed on the call:

- The company is on-track to report Phase II data from the antidote + Xarelto and the antidote + Lovenox trials over the next two quarters. PTLA plans to start the antidote + edoxaban study in collaboration with Daiichi Sankyo in 2014.
- PTLA will submit the final Phase III protocols to the FDA by YE:13 and anticipates that the Phase III trials will start in H1:14. The Phase IV outcomes study is expected to start in 2014, pending final discussion with the FDA on endpoints and populations. The company expects the trial will need hundreds of patients, not thousands.
- Data from the Phase III trial(s) are expected in 2015.

### Update Data for a Competitive Program

At the upcoming AHA meeting, privately-held Perosphere will present its first-in-human Phase I experience for its Factor Xa reversal agent. PER977 is a small molecule and if safe and effective, it could have the advantage of lower COGS (small molecules vs biologics) and easier storage by hospitals (e.g. longer shelf-life, no freezing or refrigeration required). It may be more technically difficult to develop a small molecule universal inhibitor of Factor Xa inhibitors, which may have divergent structures.

Perosphere is currently running an 80 patient Phase I trial of their anticoagulant reversal agent, PER977, which they believe is also a universal reversal agent, similar to PRT4445. The first trial is PER977+edoxaban and is being run in collaboration with Daiichi Sankyo. At EHA, the company is presenting data on this program, which we believe could be the first human data for this program.

### Oral session, Mon Nov 18

**“A synthetic small molecule which reverses over-dosage and bleeding by the new oral anticoagulants”**

## Betrixaban Phase III APEX Trial Readout Due in 2015

PTLA is testing betrixaban in acute medically ill patients in a Phase III outcomes trial (APEX) in 6,850 patients. The trial started in 2012, and enrollment is expected to complete in mid-2015 with data in Q3:15.

### Overview of pivotal APEX trial:

The APEX trial randomizes patients to either the current standard of care Lovenox or betrixaban. Patients in the Lovenox arm receive subcutaneous injections for 10 days (+/- 4 days) while in the hospital, and patients in the betrixaban arm receive daily oral betrixaban both in the hospital and after discharge for a total of 35 days.

The APEX trial is powered to show 35% reduction in risk of clots, which is based on learning from prior trials of competitor agents, the unique properties of betrixaban, and changes to the trial design relative to competitor trials, which should increase the overall probability of success. The trial is enriched for patients at elevated risk for clots after discharge- over age 75 and having elevated levels of D-dimer.

## PRT2070: An Emerging Drug for BCR Failure patients

Portola has begun Phase I testing of PRT2070, a dual SYK and JAK inhibitor (previously announced) in CLL and NHL. SYK is a key component of the BCR pathway, and JAK has been implicated in cytokine signaling with proven therapeutic potential in hematologic disease.

**Exhibit 1: PTLA News Flow**

Timing	Expected News Flow	Program
<b>Andexanet alfa (PRT4445) - Factor Xa antidote</b>		
Q4:13	Additional Phase II data (Xarelto reversal)	PRT4445
Q4:13/Q1:14	Additional Phase II data (enoxaparin reversal)	PRT4445
H1:14	Phase III First-Patient-In	PRT4445
Late 2015/2016	BLA filing	PRT4445
<b>Betrixaban - Factor Xa inhibitor</b>		
2014	Futility analysis	Betrixaban
Mid:15	Complete enrollment	Betrixaban
Q3:15	Phase III Data	Betrixaban
<b>PRT2070 - SYK/JAK inhibitor</b>		
2014	Phase I Proof-of-Concept Data	PRT2070
Mid:15	Phase II Proof-of-Concept Data	PRT2070

Source: Company data, Credit Suisse estimates

**Exhibit 2: PTLA Earnings Model**

	2011A	2012A	Q1:13A	Q2:13A	Q3:13A	Q4:13E	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E
US sales (prob. adjusted)														
Betrixaban										29.1	151.2	212.3	271.6	336.2
Andexanet alfa (PRT4445)										25.0	67.3	91.3	123.3	160.0
Total US sales										54.1	218.5	303.6	394.9	496.2
Royalty revenue										4.3	22.3	39.5	56.6	79.3
Collaboration and license revenue	78.0	72.0	3.1	2.6	2.8		8.5		65.0					
<b>Total revenue</b>	<b>78.0</b>	<b>72.0</b>	<b>3.1</b>	<b>2.6</b>	<b>2.8</b>		<b>8.5</b>		<b>65.0</b>	<b>58.4</b>	<b>240.8</b>	<b>343.2</b>	<b>451.5</b>	<b>575.5</b>
Cost of goods sold										29.1	54.6	62.0	72.7	81.9
Milestone expense									(2.0)	(13.0)				
R&D	46.1	49.7	17.7	20.8	18.1	27.5	84.1	105.6	93.7	70.1	65.3	65.5	70.7	75.8
SG&A	12.1	11.5	3.0	3.7	3.9	3.9	14.6	16.2	18.7	87.2	80.0	83.6	88.7	94.3
<b>Total operating expenses</b>	<b>58.2</b>	<b>61.2</b>	<b>20.8</b>	<b>24.5</b>	<b>22.0</b>	<b>31.4</b>	<b>98.7</b>	<b>121.8</b>	<b>110.5</b>	<b>144.3</b>	<b>145.3</b>	<b>149.1</b>	<b>159.4</b>	<b>170.1</b>
Income (loss) from operations	19.9	10.9	(17.7)	(21.9)	(19.2)	(31.4)	(90.2)	(121.8)	(45.5)	(114.9)	40.9	132.1	219.5	323.5
Interest and other income, net	0.1	0.5	(0.5)	0.3	0.7	0.3	0.8	0.7	0.3	0.3	0.2	0.3	0.5	1.0
Interest expense	(0.0)													
Income (loss) before income taxes	20.0	11.4	(18.1)	(21.6)	(18.6)	(31.1)	(89.4)	(121.1)	(45.1)	(114.7)	41.0	132.4	220.0	324.5
Provision for income taxes											0.8	2.6	4.4	64.7
<b>Net income (loss)</b>	<b>20.0</b>	<b>11.4</b>	<b>(18.1)</b>	<b>(21.6)</b>	<b>(18.6)</b>	<b>(31.1)</b>	<b>(89.4)</b>	<b>(121.1)</b>	<b>(45.1)</b>	<b>(114.7)</b>	<b>40.2</b>	<b>129.8</b>	<b>215.6</b>	<b>259.8</b>
<b>Diluted EPS - (proforma)</b>		<b>\$0.44</b>	<b>(\$0.71)</b>	<b>(\$1.47)</b>	<b>(\$0.53)</b>	<b>(\$0.86)</b>	<b>(\$3.20)</b>	<b>(\$3.01)</b>	<b>(\$0.98)</b>	<b>(\$2.47)</b>	<b>\$0.78</b>	<b>\$2.50</b>	<b>\$4.12</b>	<b>\$4.91</b>
Basic		25.4	25.4	14.7	35.2	36.4	27.9	40.3	46.0	46.4	46.9	47.4	47.8	48.3
Diluted		26.1	25.4	14.7	2,018.0	40.3	524.6	44.4	50.3	50.8	51.3	51.8	52.3	52.9

Source: Company data, Credit Suisse estimates

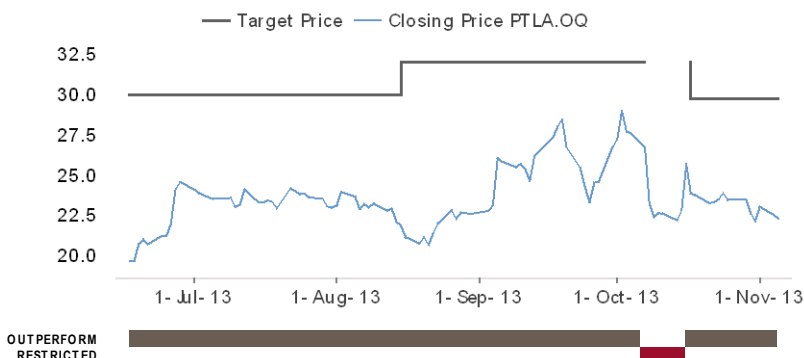
**Companies Mentioned** (Price as of 05-Nov-2013)**Daiichi Sankyo** (4568.T, ¥1,834)**Portola Pharmaceuticals, Inc.** (PTLA.OQ, \$22.32, OUTPERFORM[V], TP \$32.0)**Disclosure Appendix****Important Global Disclosures**

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**3-Year Price and Rating History for Portola Pharmaceuticals, Inc. (PTLA.OQ)**

PTLA.OQ	Closing Price	Target Price	
Date	(US\$)	(US\$)	Rating
17-Jun-13	19.68	30.00	O *
15-Aug-13	21.86	32.00	
07-Oct-13	26.74		R
17-Oct-13	25.68	29.74	O

\* Asterisk signifies initiation or assumption of coverage.



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Underperform/Sell*	15%	(40% banking clients)
Restricted	3%	

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#### Price Target: (12 months) for Portola Pharmaceuticals, Inc. (PTLA.OQ)

**Method:** Our \$32 target for PTLA is derived using a probability-adjusted DCF, assigning a 65% probability of success to betrixaban and a 55% probability to PRT4445. We use a 12% discount rate and model through the entire lifecycle. We expect the company to be cash flow positive in 2017.

**Risk:** Risks to our \$32 target price for PTLA are (1) betrixaban Phase III APEX study fails to demonstrate clinical significance, (2) one or more factor Xa products enter the Clinically III market before betrixaban can capture a meaningful market share, (3) unexpected regulatory hurdles for PTLA's antidote program, (4) potential competition from other factor Xa antidote products, and (5) financing risk with a future capital raise.

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