

Equity Research

February 28, 2014

Price: \$26.65 (02/27/2014)

Price Target: \$45.00

OUTPERFORM (1)

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Key Data

Symbol	NASDAQ: PTLA
52-Week Range:	\$30.95 - 14.50
Market Cap (MM):	\$1,058.3
Net Debt (MM):	\$(319.0)
Cash/Share:	NA
Dil. Shares Out (MM):	39.5
Enterprise Value (MM):	\$883.5
ROIC:	NA
ROE (LTM):	NA
BV/Share:	\$5.62
Dividend:	NA

FY (Dec)	2013A	2014E	2015E
Earnings Per Share			
Q1	\$(0.71)	\$(0.69)	-
Prior Q1	-	\$(0.60)	-
Q2	\$(1.47)	\$(0.82)	-
Prior Q2	-	\$(0.66)	-
Q3	\$(0.53)	\$(0.95)	-
Prior Q3	-	\$(0.68)	-
Q4	\$(0.63)	\$(1.15)	-
Prior Q4	\$(0.56)	\$(0.71)	-
Year	\$(2.90)	\$(3.60)	\$(4.70)
Prior Year	\$(2.80)	\$(2.65)	\$(3.55)
P/E	NM	NM	NM
Consensus EPS	\$(2.90)	\$(2.84)	\$(2.73)
Prior Year	\$(3.21)	-	-

Consensus source: Thomson Reuters

Revenue (MM)

Year	\$10.5	\$10.0	\$10.0
Prior Year	\$11.0	-	-
EV/S	84.1x	88.4x	88.4x

Earnings Update

Reports Q4; Andexanet On Track To Produce Pivotal Data By YE:14

The Cowen Insight

Andexanet alfa's pivotal trials will begin in H1:14. We think few investors appreciate that the first pivotal data will be released before the end of 2014, meaning that PTLA shares have a relatively low risk near-term catalyst, in our opinion. We continue to think Portola is undervalued and consider it a top small cap idea.

Design Of Andexanet Alfa's Pivotal Trials Crystallizing.

As a breakthrough therapy, andexanet benefited from FDA's guidance on the design of the Phase III program. Portola will conduct two trials, one with Eliquis and a second with Xarelto. Both will assess the andexanet's ability to neutralize the Factor Xa inhibitors via pharmacodynamic markers such as anti-Factor Xa levels, plasma free fraction of anticoagulant, and thrombin generation. Each trial will have two parts, with each part enrolling 35 healthy volunteers. In part one, a bolus dose of andexanet will be given. In part two, patients will be administered a bolus plus infusion of andexanet. Portola expects andexanet will have 2 bolus doses, and 2 infusion doses, to use against each Factor Xa. Portola expects the two doses will neutralize the full range of Factor Xa doses. While the specific andexanet doses have not been disclosed, we expect them to be in the range of those used in Phase II. Portola expects to release data from the bolus portions of both the Eliquis and Xarelto trials by the end of 2014, with data from the infusion portions expected during H1:15. As andexanet's Phase II data show it can rapidly reverse the effects of Eliquis and Xarelto, we think the program is de-risked and that the chances of success in the pharmacodynamic marker Phase III trials is very high. Portola expects to file a BLA for andexanet by the end of 2015. Portola is working to design a confirmatory Phase IV trial for andexanet. Portola expects the trial to be conducted in either patients with a major bleed, or those undergoing surgery. Portola anticipates initiating the trial by YE:14 or early in 2015, and notes that andexanet's BLA filing may be dependent upon the confirmatory trial achieving a prespecified level of enrollment.

Betrixaban's Enrollment Back On Pace.

Betrixaban APEX study has enrolled over 30% of its target patients at over 400 global trial sites. Management noted that enrollment lagged for a time as logistical issues prevented a number of overseas sights from opening on time. This has delayed APEX's interim futility analysis (to early 2015) and enrollment completion (to YE:15) by 3-6 months. Portola is pleased with the quality of patients that have been enrolled, noting that the trial's pooled, blinded event rate is consistent with PTLA's expectations.

Q4 Financials and 2014 Guidance

Portola reported a Q4 loss of \$25.1MM, or \$0.63/share, and ended Q4 with \$319MM in cash. Portola is funded through pivotal data releases for both Betrixaban and andexanet. PTLA provided 2014 guidance. With multiple ongoing trials and a few about to commence, operating expenses will be between \$145-\$160MM. Portola expects to end 2014 with approximately \$185-\$200MM in cash. We have adjusted our estimates to be consistent.

Please see addendum of this report for important disclosures.

At A Glance

Our Investment Thesis

Betrixaban, a once-daily Factor Xa inhibitor, has best-in-class pharmacokinetic and pharmacodynamics properties. This profile combined with an intelligent trial design, make us hopeful for the success of the Phase III APEX study. Following an interim futility analysis in early 2015, final results are expected in 2016. We project Betrixaban will achieve \$700MM in revenue by 2020. Portola's second candidate is andexanet alfa (PRT4445), a Factor Xa decoy that is in Phase II as an antidote to direct and indirect Factor Xa inhibitors. We expect it to enter an abbreviated pivotal program in H1:14, and to be launched in 2016. We project it will achieve \$355MM in WW revenue in 2020. Our analysis suggests that Portola is undervalued based on the potential of andexanet and Betrixaban. We believe that at current levels investors are being more than adequately compensated for the clinical, regulatory, and commercial risk associated with the development of Portola's pipeline.

Forthcoming Catalysts

- Initiate pivotal trial of andexanet alfa ('4445)
- Report data from andexanet's Phase III trial
- Interim futility analysis in Betrixaban's APEX trial

Base Case Assumptions

- Andexanet alfa approved based on biomarker data
- Andexanet alfa has sales of \$355 in 2018 and is patent protected to 2029
- Betrixaban achieves \$700MM revenue by 2020 and patent expires 2026
- Betrixaban presents best-in-class pharmacokinetics and pharmacodynamics properties

Upside Scenario

- At least one other pipeline product succeeds and creates shareholder value
- Sales of Andexanet alfa surpass expectations
- Betrixaban exceeds \$700MM in revenue by 2020

Downside Scenario

- Andexanet alfa's development stumbles
- Market for andexanet alfa does not materialize
- Betrixaban is not successfully developed

Price Performance



Source: Bloomberg

Company Description

Portola Pharmaceuticals is a biopharmaceutical company focused on the development of novel therapies in the areas of thrombosis, hematology, and inflammation. Portola's first candidate is Betrixaban, a once-daily Factor Xa inhibitor that is in the Phase III APEX trial for extended duration prophylaxis of venous thromboembolism (VTE) in acute medically ill patients. Portola's second candidate is andexanet alfa (PRT4445), a Factor Xa decoy that is in Phase II as an antidote to direct and indirect Factor Xa inhibitors. Given the unmet need for a Factor Xa antidote, andexanet received breakthrough designation in November 2013. We expect it to enter an abbreviated pivotal trial in H1:14, and to be launched in 2016. Behind Betrixaban and andexanet alfa are cerdulatinib (PRT2070), a dual Syk/JAK inhibitor for hematologic malignancies that entered the clinic in December 2013, and PRT2607, a Syk inhibitor partnered with Biogen that could move into Phase II for allergic asthma in 2014.

Analyst Top Picks

	Ticker	Price (02/27/2014)	Price Target	Rating
BioMarin Pharmaceutical	BMRN	\$83.28	\$95.00	Outperform
Gilead Sciences	GILD	\$83.65	\$95.00	Outperform
Portola Pharmaceuticals	PTLA	\$26.65	\$45.00	Outperform

Investment Thesis

Portola Pharmaceuticals is a biopharmaceutical company focused on the development of novel therapies in the areas of thrombosis, hematology, and inflammation. Portola's first candidate is Betrixaban, a once-daily Factor Xa inhibitor that is in the Phase III APEX trial for extended duration prophylaxis of venous thromboembolism (VTE) in acute medically ill patients. Betrixaban has been shown to have best-in-class pharmacokinetic and pharmacodynamics properties which, combined with an intelligent trial design, make us hopeful for the success of the APEX study. Following an interim futility analysis in early 2015, final results from the trial are expected in 2016, and we project Betrixaban will achieve \$700MM in revenue by 2020. Portola's second candidate is andexanet alfa (PRT4445), a Factor Xa decoy that will enter Phase III during H1:14 as an antidote to direct and indirect Factor Xa inhibitors. Andexanet alfa has generated promising Phase II proof-of-concept data which have shown it to safely, potently, and rapidly neutralize anti-Factor Xa activity of apixaban and rivaroxaban. Given the unmet need for a Factor Xa antidote, andexanet received breakthrough designation in November 2014. We expect the first data from its pivotal trials to be released by the end of 2014, and for it to be launched in 2016. We project andexanet alfa will achieve \$355MM in worldwide revenue in 2020. Behind Betrixaban and andexanet alfa are cerdulatinib (PRT2070), a dual Syk/JAK inhibitor for hematologic malignancies that entered the clinic in December 2013, and PRT2607, a Syk inhibitor partnered with Biogen that could move into Phase II for allergic asthma as early as this year. Our analysis suggests that Portola is undervalued based on the potential of andexanet alfa and Betrixaban. We believe that at current levels investors are being more than adequately compensated for the clinical, regulatory, and commercial risk associated with the development of Portola's pipeline.

Upcoming Milestones

Event	Timing
Initiate Phase III studies of andexanet alfa with Eliquis and XARELTO	H1:14
Data from Phase II studies of andexanet alfa and Edoxaban or Enoxaparin	Mid 2014
Proof-of-activity data from Phase I of the Phase I/II trial of cerdulatinib in patients with refractory non-Hodgkin lymphoma and chronic lymphocytic leukemia	Mid 2014
Report bolus dosing Phase III data from the studies of andexanet alfa with Eliquis and XARELTO	Q4:2014
Initiate Phase IV confirmatory study of andexanet alfa	YE:14
Conduct third planned Data Safety Monitoring Committee review of the APEX Study	2014
Report Phase II data of andexanet with additional Factor Xa inhibitors	2014
Continue to advance commercial-scale manufacturing at Lonza Group Ltd.	2014
Interim futility analysis in Betrixaban's APEX trial	Early 2015
Report bolus + infusion data from the Phase III studies of andexanet alfa with Eliquis and XARELTO	H1:15
Finish enrollment in Betrixaban's APEX trial	YE:15
File a BLA for conditional approval of andexanet alfa	YE:15
Data from cerdulatinib's Phase II proof of concept trial, including data in hematologic cancer patients with genetically-defined tumors	H2:15

Source: Cowen and Company

Portola Pharmaceuticals Inc. Quarterly P&L (\$MM)

	Q1:13A	Q2:13A	Q3:13A	Q4:13A	2013A	Q1:14E	Q2:14E	Q3:14E	Q4:14E	2014E
Betrixaban	-	-	-	-	-	-	-	-	-	-
PRT4445	-	-	-	-	-	-	-	-	-	-
Collaboration and Licensing Revenue	3.1	2.6	2.8	2.1	10.5	2.5	2.5	2.5	2.5	10.0
Other	-	-	-	-	-	-	-	-	-	-
Total Revenue	3.1	2.6	2.8	2.1	10.5	2.5	2.5	2.5	2.5	10.0
COGS	-	-	-	-	-	-	-	-	-	-
<i>Gross Margin</i>										
R&D	17.7	20.8	18.1	22.6	79.3	25.0	30.0	35.0	40.0	130.0
SG&A	3.0	3.7	3.9	4.8	15.4	5.0	5.5	6.0	9.0	25.5
Other	-	-	-	-	-	-	-	-	-	-
Operating Expenses	20.8	24.5	22.0	27.4	94.7	30.0	35.5	41.0	49.0	155.5
Operating Income / (Loss)	(17.7)	(21.9)	(19.2)	(25.4)	(84.2)	(27.5)	(33.0)	(38.5)	(46.5)	(145.5)
Interest Income, net	(0.5)	0.3	0.7	0.3	0.8	0.1	0.1	0.2	0.2	0.5
Other Income	-	-	-	-	-	-	-	-	-	-
Pretax net income	(18.1)	(21.6)	(18.6)	(25.1)	(83.4)	(27.4)	(32.9)	(38.4)	(46.4)	(145.0)
Taxes	-	-	-	-	-	-	-	-	-	-
<i>Tax Rate</i>	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%
GAAP Net Income to common stockholders	(18.1)	(21.6)	(18.6)	(25.1)	(83.4)	(27.4)	(32.9)	(38.4)	(46.4)	(145.0)
GAAP EPS	\$ (0.71)	\$ (1.47)	\$ (0.53)	\$ (0.63)	\$ (2.90)	\$ (0.69)	\$ (0.82)	\$ (0.95)	\$ (1.15)	\$ (3.60)
Diluted Shares Outstanding (MM)	25.5	14.7	35.2	39.5	28.7	40.0	40.2	40.3	40.4	40.2

Source: Cowen and Company

Portola Pharmaceuticals Inc. Annual P&L (\$MM)

	2013A	2014E	2015E	2016E	2017E	2018E	2019E	2020E
Betrixaban	-	-	-	-	150.0	300.0	475.0	700.0
PRT4445	-	-	-	50.0	120.0	190.0	275.0	355.0
Collaboration and Licensing Revenue	10.5	10.0	10.0	10.0	10.0	10.0	10.0	10.0
Other	-	-	-	-	-	-	-	-
Total Revenue	10.5	10.0	10.0	60.0	280.0	500.0	760.0	1,065.0
COGS	-	-	-	14.9	44.4	74.3	110.4	151.4
<i>Gross Margin</i>				70%	84%	85%	85%	86%
R&D	79.3	130.0	150.0	165.0	180.0	190.0	200.0	210.0
SG&A	15.4	25.5	51.3	151.5	205.5	250.0	275.0	300.0
Other	-	-	-	-	-	-	-	-
Operating Expenses	94.7	155.5	201.3	331.4	429.9	514.3	585.4	661.4
Operating Income / (Loss)	(84.2)	(145.5)	(191.3)	(271.4)	(149.9)	(14.3)	174.6	403.6
Interest Income, net	0.8	0.5	1.0	1.0	1.0	1.0	7.0	12.5
Other Income	-	-	-	-	-	-	-	-
Pretax net income	(83.4)	(145.0)	(190.3)	(270.4)	(148.9)	(13.3)	181.6	416.1
Taxes	-	-	-	-	-	-	-	-
<i>Tax Rate</i>	-	-	-	-	-	-	0%	0%
GAAP Net Income to common stockholders	(83.4)	(145.0)	(190.3)	(270.4)	(148.9)	(13.3)	181.6	416.1
GAAP EPS	(2.90)	(3.60)	(4.70)	(6.60)	(3.55)	(0.30)	3.95	8.85
Diluted Shares Outstanding (MM)	28.7	40.2	40.5	41.0	42.0	45.0	46.0	47.0

Source: Cowen and Company

Portola DCF Analysis

Financial Year End	12/31/2012	Portola: DCF Valuation																		
Valuation Date	2/27/2014																			
Discount Rate	10.0%																			
Terminal Growth Rate	-20.0%																			
\$MM		2013	2014	2015	2016	2017	2018	2019	2020	2021	2022	2023	2024	2025	2026	2027	2028	2029	2030	2031
Betrixaban		0	0	0	0	150	300	475	700	840	966	1,063	1,116	1,172	644	290	29	26	23	21
Growth (%)							100%	58%	47%	20%	15%	10%	5%	5%	-45%	-55%	-90%	-10%	-10%	-10%
PRT4445		0	0	0	50	120	190	275	355	426	490	549	593	622	653	666	700	714	728	743
Growth (%)						140%	58%	45%	29%	20%	15%	12%	8%	5%	5%	2%	2%	2%	2%	2%
Collaboration and Licensing Revenue		11	10	10	10	10	10	10	10	10	10	10	10	10	10	10	10	10	10	10
Growth (%)																				
Other		0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0	0
Growth (%)																				
Total Revenues		11	10	10	60	280	500	760	1065	1276	1466	1621	1718	1804	1308	986	739	750	761	774
Growth (%)						367%	79%	52%	40%	20%	15%	11%	6%	5%	-28%	-25%	-25%	2%	2%	2%
COGS		0	0	0	15	44	74	110	151	187	215	239	261	275	235	223	212	216	220	224
COGS as a % of sales					30%	16%	15%	15%	14%	15%	15%	15%	15%	15%	18%	23%	29%	29%	29%	29%
R&D		79	130	150	165	180	190	200	210	153	176	162	172	144	105	49	37	37	38	39
R&D as a % of Revenues					275%	64%	38%	26%	20%	12%	12%	10%	10%	8%	8%	5%	5%	5%	5%	5%
SG&A		15	26	51	152	206	250	275	300	319	337	324	344	325	235	148	111	112	114	116
SG&A as a % of Revenues					253%	73%	50%	36%	28%	25%	23%	20%	20%	18%	18%	15%	15%	15%	15%	15%
Operating Income		-64	-146	-191	-271	-150	-14	175	404	617	736	896	941	1060	733	566	379	364	369	395
Tax		0	0	0	0	0	0	0	0	93	148	269	329	371	257	198	133	134	136	138
Tax rate		0%	0%	0%	0%	0%	0%	0%	0%	15%	20%	30%	35%	35%	35%	35%	35%	35%	35%	35%
NOL/Tax Assets Utilized																				
Tax rate																				
Taxes Paid		0	0	0	0	0	0	0	0	93	148	269	329	371	257	198	133	134	136	138
Approx Free Cash Flow		(64)	(146)	(191)	(271)	(150)	(14)	175	404	525	591	627	612	689	476	368	247	250	253	257
Years		-0.16	0.84	1.84	2.84	3.84	4.84	5.84	6.84	7.84	8.84	9.84	10.84	11.84	12.84	13.84	14.84	15.84	16.84	17.84
Discount Factor		1.02	0.92	0.84	0.76	0.69	0.63	0.57	0.52	0.47	0.43	0.39	0.36	0.32	0.29	0.27	0.24	0.22	0.20	0.18
NPV of Cash flows		(65)	(134)	(161)	(207)	(104)	(9)	100	210	249	254	246	218	223	140	98	60	55	51	47
Terminal Value Calculation																				
Final year FCF	257																			
Perpetual Growth Rate	-20.0%																			
Terminal Value	685																			
Discount Factor	0.18																			
Present Value of Terminal Value	125																			
Present Value of Cash Flows	1,336																			
Enterprise Value	1,461																			
Add: Net cash	319																			
Market Value	1,780																			
Fully Diluted Shares Outstanding	39.5																			
Value per Fully Diluted Share	\$45.10																			

Source: Cowen and Company.

Valuation Methodology And Risks

Valuation Methodology

Biotechnology:

In calculating our 12-month target price, we employ one or more valuation methodologies, which include a discounted earnings analysis, discounted cash flow analysis, net present value analysis and/or a comparable company analysis. These analyses may or may not require the use of objective measures such as price-to-earnings or price-to-sales multiples as well as subjective measures such as discount rates.

We make investment recommendations on early stage (pre-commercial) biotechnology companies based upon an assessment of their technology, the probability of pipeline success, and the potential market opportunity in the event of success. However, because these companies lack traditional financial metrics, we do not believe there are any good methodologies for assigning a specific target price to such stocks.

Investment Risks

Biotechnology:

There are multiple risks that are inherent with an investment in the biotechnology sector. Beyond systemic risk, there is also clinical, regulatory, and commercial risk. Additionally, biotechnology companies require significant amounts of capital in order to develop their clinical programs. The capital-raising environment is always changing and there is risk that necessary capital to complete development may not be readily available.

Risks To The Price Target

Much of Portola's valuation rests on the potential of two candidates, Betrixaban and PRT4445. Projecting future sales for any product is difficult, and this is particularly the case for candidates that are still in clinical development. Portola's stock could be impacted by changes in the regulatory, commercial, or competitive environment for either. In particular, Betrixaban is in development in an indication for which candidates of the same mechanism have failed clinical trials; Betrixaban's clinical development must therefore be considered risky. The studies necessary for PRT4445 to receive regulatory approval have yet to be defined; there is therefore risk that its clinical development takes longer than we anticipate.

Addendum

Stocks Mentioned In Important Disclosures

Ticker	Company Name
BMRN	BioMarin Pharmaceutical
GILD	Gilead Sciences
PTLA	Portola Pharmaceuticals

Analyst Certification

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COWEN AND COMPANY RATING DEFINITIONS

Cowen and Company Rating System effective May 25, 2013

Outperform (1): The stock is expected to achieve a total positive return of at least 15% over the next 12 months

Market Perform (2): The stock is expected to have a total return that falls between the parameters of an Outperform and Underperform over the next 12 months

Underperform (3): Stock is expected to achieve a total negative return of at least 10% over the next 12 months

Assumption: The expected total return calculation includes anticipated dividend yield

Cowen and Company Rating System until May 25, 2013

Outperform (1): Stock expected to outperform the S&P 500

Neutral (2): Stock expected to perform in line with the S&P 500

Underperform (3): Stock expected to underperform the S&P 500

Assumptions: Time horizon is 12 months; S&P 500 is flat over forecast period

Cowen Securities, formerly known as Dahlman Rose & Company, Rating System until May 25, 2013

Buy – The fundamentals/valuations of the subject company are improving and the investment return is expected to be 5 to 15 percentage points higher than the general market return

Sell – The fundamentals/valuations of the subject company are deteriorating and the investment return is expected to be 5 to 15 percentage points lower than the general market return

Hold – The fundamentals/valuations of the subject company are neither improving nor deteriorating and the investment return is expected to be in line with the general market return

Cowen And Company Rating Definitions

Distribution of Ratings/Investment Banking Services (IB) as of 12/31/13

Rating	Count	Ratings Distribution	Count	IB Services/Past 12 Months
Buy (a)	415	59.20%	68	16.39%
Hold (b)	270	38.52%	4	1.48%
Sell (c)	16	2.28%	1	6.25%

(a) Corresponds to "Outperform" rated stocks as defined in Cowen and Company, LLC's rating definitions. (b) Corresponds to "Market Perform" as defined in Cowen and Company, LLC's ratings definitions. (c) Corresponds to "Underperform" as defined in Cowen and Company, LLC's ratings definitions.

Note: "Buy", "Hold" and "Sell" are not terms that Cowen and Company, LLC uses in its ratings system and should not be construed as investment options. Rather, these ratings terms are used illustratively to comply with FINRA and NYSE regulations.

Portola Pharmaceuticals Rating History as of 02/27/2014

powered by: BlueMatrix



BioMarin Pharmaceutical Rating History as of 02/27/2014

powered by: BlueMatrix



Gilead Sciences Rating History as of 02/27/2014

powered by: BlueMatrix



Legend for Price Chart:

I = Initiation | 1 = Outperform | 2 = Market Perform | 3 = Underperform | UR = Price Target Under Review | T = Terminated Coverage | \$xx = Price Target | NA = Not Available

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