

Equity Research

May 13, 2014

Price: \$23.95 (05/12/2014)

Price Target: \$45.00

OUTPERFORM (1)

Phil Nadeau, Ph.D.

646.562.1336

phil.nadeau@cowen.com

Cristina Ghenoiu, Ph.D.

646.562.1401

cristina.ghenoiu@cowen.com

Key Data

Symbol	NASDAQ: PTLA
52-Week Range:	\$30.95 - 14.50
Market Cap (MM):	\$984.2
Net Debt (MM):	\$(319.0)
Cash/Share:	\$6.57
Dil. Shares Out (MM):	39.5
Enterprise Value (MM):	\$715.5
ROIC:	NA
ROE (LTM):	NA
BV/Share:	\$7.24
Dividend:	NA

FY (Dec)	2013A	2014E	2015E
Earnings Per Share			
Q1	\$(0.71)	\$(0.75)A	-
Prior Q1	-	\$(0.69)	-
Q2	\$(1.47)	\$(0.82)	-
Prior Q2	-	-	-
Q3	\$(0.53)	\$(0.95)	-
Prior Q3	-	-	-
Q4	\$(0.63)	\$(1.15)	-
Prior Q4	-	-	-
Year	\$(2.90)	\$(3.66)	\$(4.70)
Prior Year	-	\$(3.60)	-
P/E	NM	NM	NM
Consensus EPS	\$(2.90)	\$(3.52)	\$(3.68)
Prior Year	-	\$(2.84)	\$(2.73)

Consensus source: Thomson Reuters

Revenue (MM)

Year	\$10.5	\$9.9	\$10.0
Prior Year	-	\$10.0	-
EV/S	68.1x	72.3x	71.6x

Earnings Update

Reports Q1; First Andexanet Ph. III Data On Track For Q4

The Cowen Insight

Portola remains well funded with \$306MM in cash. The first data from Andexanet's' Ph. 3 program is on track for Q4:14. Based on its strong Ph. 2 data, we think Andexanet is very likely to succeed in Ph. 3, and enjoy a solid market opportunity. We continue to think Portola is undervalued and consider it a top small cap idea.

Andexanet's Phase 3 Trial With Xarelto Initiated.

Portola has initiated Phase III trials of Andexanet Alfa and the factor Xa inhibitors Eliquis and XARELTO. Both are randomized, double-blind, placebo-controlled studies enrolling healthy volunteers (ages 50-75 years). Each trial will have two parts and will assess the efficacy of neutralization via pharmacodynamics markers such as anti-Factor Xa levels, plasma free fraction of anticoagulant, and thrombin generation. Although the initiation of the Eliquis trial had been previously disclosed, the initiation of the Xarelto trial was announced for the first time last night.

Based on its compelling Ph. 2 data, we think Andexanet's chances of Phase III success are very high. Data from the bolus portion of the Xarelto and Eliquis studies will be available in Q4:14 while data from the bolus and infusion portion will be released in H1:15. The Phase 3b/4 confirmatory study is expected to initiate in late 2014 or early 2015.

Andexanet's Manufacturing Strategy Adjusted.

The clinical supply of andexanet alfa is produced by CMC Biologics. Portola is in an agreement with the Lonza Group for commercial scale-up and manufacturing. Thus far the material produced by the Lonza Group has shown comparability with the CMC batches of andexanet, but Lonza is lagging behind the timelines necessary for a 2015 BLA submission. CMC Biologics will now produce commercial supply for the BLA filing and U.S. launch, while Lonza material will enter the market several months later. This strategy somewhat de-risks the filing timelines. However, there is a chance that if the andexanet launch is particularly fast, it could be supply constrained for one or two quarters. While not ideal, we think a fast launch in which demand outstrips supply for a few months would be considered a good problem to have by investors.

The Rest Of The Pipeline Makes Good Progress.

In March the Data Safety Monitoring Committee held its third planned review of the Betrixaban APEX study and advised the trial to proceed as planned. APEX is 40% enrolled and on track to complete enrollment by the end of 2015. A futility analysis is likely in early 2015. Detailed data from the Phase I dose escalation study of Cerdulatinib is expected later in the year.

Q1 Financials.

Portola reported a Q1 loss of \$30.7MM, or \$0.75/share and ended Q1 with \$306MM in cash. Portola continues to be funded through pivotal data releases for both Betrixaban and andexanet.

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. The first Ph. 3 data will be released in Q4:14, and we expect it 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

- Report data from andexanet's Phase III trials
- 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 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 (05/12/2014)	Price Target	Rating
BioMarin Pharmaceutical	BMRN	\$59.30	\$95.00	Outperform
Gilead Sciences	GILD	\$80.35	\$95.00	Outperform
Portola Pharmaceuticals	PTLA	\$23.95	\$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 has entered a Phase III program aiming to establish it 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 2013. 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
Data from Phase II studies of andexanet alfa Enoxaparin	June 25, ISTH
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 additional planned Data Safety Monitoring Committee reviews of the APEX Study	2014
Data from Phase II studies of andexanet alfa and Edoxaban	2014
Report Phase II data of andexanet with additional Factor Xa inhibitors	2014
Continue to advance commercial-scale manufacturing at Lonza Group Ltd.	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	Late 2014
Initiate a Phase 3b/4 confirmatory study for andexanet alfa	Late 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	Q1:14A	Q2:14E	Q3:14E	Q4:14E	2014E
Betrixaban	-	-	-	-	-	-	-	-	-	-
PRT4445	-	-	-	-	-	-	-	-	-	-
Collaboration and Licensing Revenue	3.1	2.6	2.8	2.1	10.5	2.4	2.5	2.5	2.5	9.9
Other	-	-	-	-	-	-	-	-	-	-
Total Revenue	3.1	2.6	2.8	2.1	10.5	2.4	2.5	2.5	2.5	9.9
COGS	-	-	-	-	-	-	-	-	-	-
<i>Gross Margin</i>										
R&D	17.7	20.8	18.1	22.6	79.3	28.2	30.0	35.0	40.0	133.2
SG&A	3.0	3.7	3.9	4.8	15.4	5.2	5.5	6.0	9.0	25.7
Other	-	-	-	-	-	-	-	-	-	-
Operating Expenses	20.8	24.5	22.0	27.4	94.7	33.4	35.5	41.0	49.0	158.9
Operating Income / (Loss)	(17.7)	(21.9)	(19.2)	(25.4)	(84.2)	(31.0)	(33.0)	(38.5)	(46.5)	(149.0)
Interest Income, net	(0.5)	0.3	0.7	0.3	0.8	0.3	0.1	0.2	0.2	0.7
Other Income	-	-	-	-	-	-	-	-	-	-
Pretax net income	(18.1)	(21.6)	(18.6)	(25.1)	(83.4)	(30.7)	(32.9)	(38.4)	(46.4)	(148.3)
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)	(30.7)	(32.9)	(38.4)	(46.4)	(148.3)
GAAP EPS	\$ (0.71)	\$ (1.47)	\$ (0.53)	\$ (0.63)	\$ (2.90)	\$ (0.75)	\$ (0.82)	\$ (0.95)	\$ (1.15)	\$ (3.66)
Diluted Shares Outstanding (MM)	25.5	14.7	35.2	39.5	28.7	41.0	40.2	40.3	40.4	40.5

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	9.9	10.0	10.0	10.0	10.0	10.0	10.0
Other	-	-	-	-	-	-	-	-
Total Revenue	10.5	9.9	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	133.2	150.0	165.0	180.0	190.0	200.0	210.0
SG&A	15.4	25.7	51.3	151.5	205.5	250.0	275.0	300.0
Other	-	-	-	-	-	-	-	-
Operating Expenses	94.7	158.9	201.3	331.4	429.9	514.3	585.4	661.4
Operating Income / (Loss)	(84.2)	(149.0)	(191.3)	(271.4)	(149.9)	(14.3)	174.6	403.6
Interest Income, net	0.8	0.7	1.0	1.0	1.0	1.0	7.0	12.5
Other Income	-	-	-	-	-	-	-	-
Pretax net income	(83.4)	(148.3)	(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)	(148.3)	(190.3)	(270.4)	(148.9)	(13.3)	181.6	416.1
GAAP EPS	(2.90)	(3.66)	(4.70)	(6.60)	(3.55)	(0.30)	3.95	8.85
Diluted Shares Outstanding (MM)	28.7	40.5	40.5	41.0	42.0	45.0	46.0	47.0

Source: Cowen and Company

Financial Year End	12/31/2012
Valuation Date	5/12/2014
Discount Rate	10.0%
Terminal Growth Rate	-20.0%

Portola: DCF Valuation																			
SMM	2013	2014	2015	2016	2017	2018	2019	2020	2021	2022	2023	2024	2025	2026	2027	2028	2029	2030	2031
Betrakaban	0	0	0	0	150	300	475	700	840	988	1,083	1,116	1,172	644	280	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	428	490	549	593	622	653	686	700	714	728	743
Growth (%)					140%	58%	45%	29%	20%	15%	12%	8%	5%	5%	5%	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	1468	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	167	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	133	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%	6%	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	-84	-149	-191	-271	-150	-14	175	404	617	738	896	941	1060	733	568	379	384	389	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	(84)	(149)	(191)	(271)	(150)	(14)	175	404	525	591	627	612	689	478	368	247	250	253	257
Years	-0.36	0.63	1.63	2.64	3.64	4.63	5.63	6.64	7.64	8.63	9.63	10.63	11.63	12.63	13.63	14.63	15.63	16.63	17.63
Discount Factor	1.04	0.94	0.86	0.78	0.71	0.64	0.58	0.53	0.48	0.44	0.40	0.36	0.33	0.30	0.27	0.25	0.23	0.20	0.19
NPV of Cash flows	(87)	(140)	(164)	(211)	(108)	(9)	102	214	253	259	250	222	227	143	100	61	56	52	48

Terminal Value Calculation	
Final year FCF	257
Perpetual Growth Rate	-20.0%
Terminal Value	685
Discount Factor	0.19
Present Value of Terminal Value	128
Present Value of Cash Flows	1,359
Enterprise Value	1,487
Add: Net cash	319
Market Value	1,806
Fully Diluted Shares Outstanding	39.5
Value per Fully Diluted Share	\$45.74

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, LLC. New York (646) 562-1000 **Boston** (617) 946-3700 **San Francisco** (415) 646-7200 **Chicago** (312) 577-2240 **Cleveland** (440) 331-3531 **Atlanta** (866) 544-7009 **London** (affiliate) 44-207-071-7500

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 03/31/14

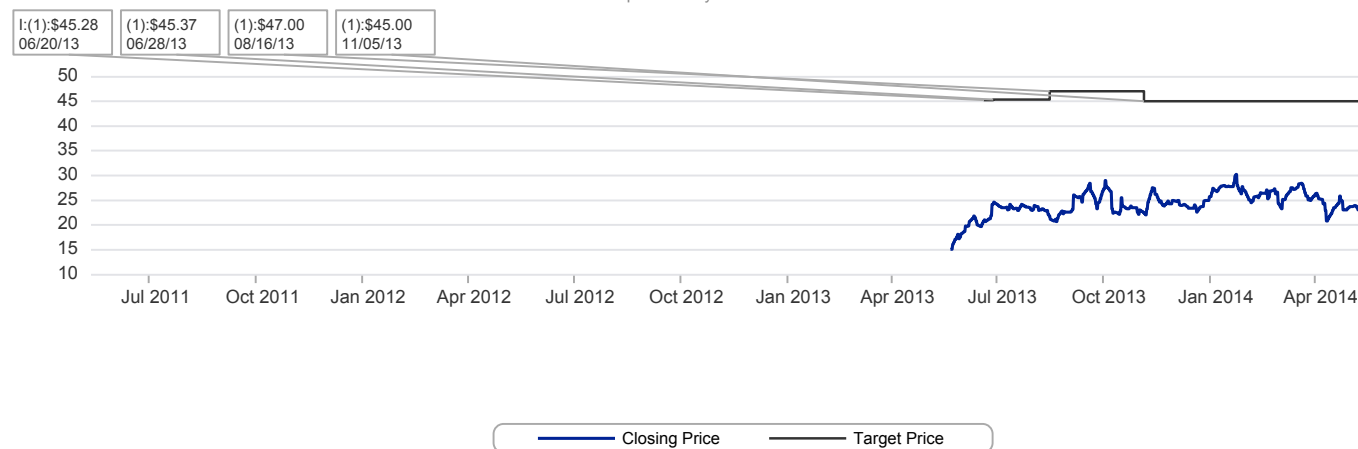
Rating	Count	Ratings Distribution	Count	IB Services/Past 12 Months
Buy (a)	407	57.08%	85	20.88%
Hold (b)	288	40.39%	8	2.78%
Sell (c)	18	2.52%	1	5.56%

(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 05/12/2014

powered by: BlueMatrix



BioMarin Pharmaceutical Rating History as of 05/12/2014

powered by: BlueMatrix



Gilead Sciences Rating History as of 05/12/2014

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 | S=Suspended

Points Of Contact

Analyst Profiles



Phil Nadeau, Ph.D.

New York

646.562.1336

phil.nadeau@cowen.com

Phil Nadeau is a senior analyst covering biotech. He has been at Cowen for 12 years. Phil holds an SB/M.Eng from MIT, and a Ph.D. from Harvard.



Cristina Ghenoiu, Ph.D.

New York

646.562.1401

cristina.ghenoiu@cowen.com

Cristina Ghenoiu is an associate covering biotech. Before joining Cowen in 2013, she was a research scientist at Rockefeller University.

Reaching Cowen

Main U.S. Locations

New York

599 Lexington Avenue
New York, NY 10022
646.562.1000
800.221.5616

Atlanta

3399 Peachtree Road NE
Suite 417
Atlanta, GA 30326
866.544.7009

Boston

Two International Place
Boston, MA 02110
617.946.3700
800.343.7068

Chicago

181 West Madison Street
Suite 1925
Chicago, IL 60602
312.577.2240

Cleveland

20006 Detroit Road
Suite 100
Rocky River, OH 44116
440.331.3531

Houston

600 Travis Street
Suite 1970
Houston, TX 77002
281.657.6800

San Francisco

555 California Street, 5th Floor
San Francisco, CA 94104
415.646.7200
800.858.9316

International Locations

Cowen International Limited

London

1 Snowden Street - 11th Floor
London EC2A 2DQ
United Kingdom
44.20.7071.7500

Cowen and Company (Asia) Limited

Hong Kong

Suite 1401 Henley Building
No. 5 Queens Road Central
Central, Hong Kong
852 3752 2333

