PTC Therapeutics, Inc (PTCT)

SMALL & MID CAP RESEARCH

Rating	**OUTPERFORM***
Price (12 Jul 13, US$)	16.94
Target price (US$)	24.00[1]
52-week price range	17.60 - 13.63
Market cap. (US$ m)	401.11
Enterprise value (US$ m)	251.79

**Stock ratings are relative to the coverage universe in each analyst's or each team's respective sector.*
[1]Target price is for 12 months.

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It Looks Like There's No Stopping Ataluren

We Are Initiating with an Outperform Rating and a $24 TP: Our bullish thesis is based on the demonstrated activity of Ataluren in two orphan genetic diseases: Duchenne muscular dystrophy (DMD) and cystic fibrosis (CF). Together, these represent a $1B market opportunity for a highly targeted patient group, not served by other drugs in development.

- **Significant Upside for Orphan Drug Companies:** Public market comps include ALXN, VRTX, BMRN, SRTA, and RNA. High drug prices, a defined path to market, and highly targeted patient populations permit a go-alone marketing strategy and increase the value of PTCT's wholly owned ataluren franchise.
- **Catalysts:** A CHMP decision on EU conditional approval is expected by year-end 2013 (not in our numbers). Two Phase III trials in DMD and CF could read out in 2015 and 2016. Advancement of PTCT's spinal muscular atrophy (SMA) program with partner Roche is expected in H2:13 and 2014.
- **Our $24 TP Is Supported by a Probability-Weighted DCF Analysis of Ataluren in DMD and CF:** We expect PTCT to hit our target ahead of the first Phase III results in mid-2015, with further upside potential from risk-lowering Phase III results, advancement of its pipeline, or potential ex-U.S. partnership(s).
- **Technology Platform with Upside Potential:** Ataluren could potentially address a subset (~10%) of multiple genetic diseases, as it targets a type of mutation, not a specific disease. PTCT's expertise in post-transcriptional regulation could drive future pipeline development (e.g., SMA).

Financial and valuation metrics

Year	12/12A	12/13E	12/14E	12/15E
EPS (CS adj.) (US$)	42.50	-2.26	-2.19	-1.64
Prev. EPS (US$)	—	—	—	—
P/E (x)	0.4	-7.5	-7.7	-10.3
P/E rel. (%)	2.5	-50.6	-57.9	-85.3
Revenue (US$ m)	33.9	30.1	20.0	27.0
EBITDA (US$ m)	-24.1	-37.6	-54.2	-55.0
OCFPS (US$)	-2.98	-2.00	-2.12	-1.31
P/OCF (x)	—	-8.5	-8.0	-12.9
EV/EBITDA (current)	-14.7	-9.4	-6.5	-6.4
Net debt (US$ m)	2	-149	-91	-229
ROIC (%)	160.91	358.28	2,118.33	1,605.00

Number of shares (m)	23.68	IC (current, US$ m)	-16.66
BV/share (Next Qtr., US$)	-19.9	EV/IC (x)	-17.5
Net debt (Next Qtr., US$ m)	-169.5	Dividend (current, US$)	—
Net debt/tot cap (Next Qtr., %)	-108.5	Dividend yield (%)	—

Source: Company data, Credit Suisse estimates

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Portfolio Manager Summary

PTCT is developing drugs to treat genetic diseases, including Duchenne muscular dystrophy (DMD) and cystic fibrosis (CF), with small molecules that target post-transcriptional gene regulation. Its 100% wholly owned lead drug ataluren targets a specific genetic defect found in approximately 13% of DMD patients and 10% of CF patients, as well as potentially in patients with multiple other genetic diseases. PTCT has established proof of efficacy in both DMD and CF. Phase III data are expected in mid-2015 for DMD and mid-2016 for CF. An earlier stage program partnered with Roche could enter the clinic in 2014 for spinal muscular atrophy (SMA).

"There's No Stopping Ataluren": This refers to the unique mechanism of action of ataluren, which causes mutated genes to produce native proteins when they would otherwise hit a premature "stop" and produce a nonfunctional truncated protein.

Exhibit 1: PTCT Pipeline

Product/Indication	Phase	Target	Partner
Ataluren - Duchenne Muscular Dystrophy	Phase III; MAA submitted	Nonsense DMD mutations	Proprietary
Ataluren - Cystic Fibrosis	Phase III ready	Class 1 CFTR Mutations	Proprietary
Spinal muscular atrophy	Preclinical	SMN2	Roche
PTC596 - Oncology	Preclinical	BMI1	Proprietary
Antibacterial	Preclinical	MDR Gram (-) bacteria	Proprietary

Source: Company data, Credit Suisse estimates.

Exhibit 2: PTCT News Flow

Timing	Event
Duchenne Muscular Distrophy	
July 2013	Submit response to 120 day questions from EMA
Sep 2013	Receive list of outstanding issues from EMA for filing
H2:13	Seek early access programs for DMD in select territories
YE 2013	Potential conditional EU approval
Mid-2014	Complete enrollment in confirmatory DMD Phase III study
H2:14	Potential data from EU open-label extension study
Mid-2015	Potential data from confirmatory DMD Phase III study
Late-2015	FDA and EMA filing for full approval
Mid-2016	Potential FDA and EMA approval
Cystic Fibrosis	
Q4:13	EMA conditional approval filing
Q1:14	Dose first patient in confimatory CF Phase III study
YE 2014	Potential conditional EU approval
Mid-2015	Complete enrollment in confirmatory CF Phase III study
Mid-2016	Potential data from confirmatory CF Phase III study
YE:16/early 2017	FDA and MAA filing for full approval
Mid-2017	Potential FDA and EMA approval
SMA program	
H2:13	Select lead compound for SMA program
2014	IND and Phase I start

Source: Company data, Credit Suisse estimates.

Investment Positives

- **Wholly Owned Late Stage Asset:** Ataluren was discovered and developed at PTC. Ataluren is currently in a Phase III trial for Duchenne muscular dystrophy (DMD), and a Phase III trial in cystic fibrosis (CF) will start dosing patients in H1:14.
- **Orphan Drug Focus:** The lead programs for ataluren are in genetically defined subsets of DMD and CF. There is significant precedence for a small company to retain global commercial rights and launch a drug in these and similarly sized markets without a partner (e.g., VRTX, ALXN, BMRN). This potential path to market provides PTCT with leverage in negotiating a partnership or potentially selling the company should it choose that route.
- **Defined Populations with Unmet Need:** The selection of patients based on specific genetic mutations lowers regulatory risk, increases pricing power, and likely translates into high commercial penetration. The particular genetic subsets targeted by ataluren are not addressed by other therapies in DMD and CF, thus reducing the competitive risk for PTCT.
- **Open-Ended Upside Potential in Other Orphan Disease:** The mechanism of action of ataluren targets a specific type of genetic mutation (nonsense mutation) and not a specific disease. For this reason, ataluren may find applications in a wide range of genetic diseases. Roughly 10% of genetic diseases are caused by nonsense mutations.
- **Significant Prior Data:** PTCT has conducted a large randomized Phase II trial in DMD and a prior Phase III trial in CF. The significant amount of data provides PTCT with a strong rationale for patient selection, dose, and endpoint assessment. The result is a lower-risk Phase III program. The abundance of data also helps investors in assessing the likelihood of clinical success.
- **Upside Potential from European Filing:** PTCT filed in October 2012 for European conditional approval in DMD based on the randomized Phase II results. PTC has received the 120-day questions and expects to respond by July, with a possible CHMP decision by year-end 2013. We have not modeled an early approval and believe that it is low probability, although it remains a potential upside trigger.
- **Scarcity Value, Partnering Potential, and Possible Take-Out Candidate:** With retained worldwide rights to ataluren, PTCT could be in an excellent position to seek a partner or other strategic options should the Phase III trial in either DMD or CF have a positive result. Current public comps suggest that a valuation of over $1B would be likely for a company with a derisked product for an orphan disease
- **Strong Cash Position:** Following the $144M IPO ($134M after underwriters discount), PTCT has pro forma cash of approximately $186M, enough to fund at least through the DMD Phase III data in H1:15 and the CF Phase III data in H2:15. Additional funding may be required if PTCT seeks to develop ataluren for other indications or if its pipeline programs advance into clinical development.
- **Pipeline:** In addition to the potential use of ataluren in other genetic diseases, PTC has several preclinical programs, which could become value drivers in the next several years. We expect a cash milestone of approximately $10M in 2013 from Roche upon selection of a candidate for preclinical tox in its SMA program. Clinical trials for the SMA program could begin in 2014, which would likely trigger additional cash milestones.
- **Platform Technology:** PTCT has expertise in a wide range of post-transcriptional areas, and this has led to the production of ataluren (nonsense mutation read-through), SMA program (splicing modification with a small molecule), and a

variety of earlier stage programs. We believe that the company can be compared to both orphan disease drug companies and other post-transcriptional modification companies.

Investment Risks

DMD Clinical and Regulatory Risk

- **Risks Associated with Primary Endpoint:** While there is significant precedence for using six-minute walk distance (6MWD) for drug approvals in other indications, this is a new regulatory path in DMD. It is also an endpoint with significant baseline variability and potential variability in the natural history of the disease. We believe that PTCT has more data on this endpoint than any other company, and the trial is designed to reduce this risk.
- **Risks Associated with Dose Selection:** PTCT has identified an active dose in Phase II to take forward in Phase III, and the company has established that higher doses can lead to lower efficacy. However, it is unclear what the "optimal" dose is or if there is a means to dose adjust for better efficacy. These uncertainties pose both clinical and regulatory risks.
- **Risks Around Label/Reimbursement:** The Phase III trial is enrolling only patients seven years or older who can walk (within specified parameters). These restrictions are designed to optimize the ability to measure the primary endpoint and are not based on safety concerns or mechanism of action. If successful, patients of all ages and walking ability would likely want to take ataluren. The breadth of the FDA/EMA-approved label and the view of payors will be important in determining the market opportunity for the drug. Our model is based on the broad utility of the drug in DMD patients with the appropriate mutations.

CF Clinical and Regulatory Risk

- **Negative Interaction with Approved Antibiotics:** PTC has demonstrated that the leading inhaled antibiotic used in CF patients (TOBI, tobramycin) antagonizes with ataluren. Consequently, use of TOBI is excluded from the planned Phase III clinical trial. The exclusion of TOBI patients could limit the market opportunity or cause some patients to go off therapy. It could also lead to infectious complications if patients opt to stay off of an active agent. (other antibiotic choices are available that do not antagonize with ataluren).
- **Prior Phase III Missed Its Endpoint:** The previously unknown interaction with TOBI was the suspected cause of the failure of the prior Phase III trial. If patients on TOBI were excluded in the final analysis, the trial would have been successful. While this is a valid analysis, it does raise some concerns.
- **Potential for More Future Competition than DMD:** CF patients need to have both copies of their gene mutated. Some patients may therefore have mutations amendable to more than one treatment modality. Cost is likely prohibitive for a patient to take two fully priced drugs. For some of these patients, competitive drugs could potentially be more efficacious.

Technology Risks

- **Mechanism of Action Has Been Called into Question:** An investigator at the University of Dundee recently published a scientific report refuting PTCT claims regarding the mechanism of action of ataluren. While we have reviewed all the available data and continue to be convinced of ataluren's activity, the presence of a

dissenting opinion among some investigators poses future headline risk and raises the concern that there are aspects of the drug that are not fully understood.

Standard Risks

- **Major Catalysts Are in 2015 and 2016:** The primary risk lowering events for PTCT are expected in mid-2015 (Phase III DMD) and mid-2016 (Phase III CF). Assuming EU conditional approval is not granted (YE 2013), then the news flow in 2014 could be light.
- **Financing Risk:** PTCT will likely need to raise substantial additional funds to seek regulatory approval and launch ataluren. Financing could be delayed if PTCT signs a commercial partner. We model a financing in Q1:15.
- **Execution Risk:** While there is precedence for a small company to commercialize orphan drugs, PTCT has never launched a product.
- **Timeline Risk:** PTCT expects to complete the Phase III DMD trial in mid-2015 and the CF trial in H2:15. Enrollment rates are projections by the company, and actual rates could differ. We believe that PTCT has been reasonably conservative, and patients with the appropriate genetic mutations will be highly motivated to seek out this trial.
- **Competition:** The competitive risks are lowered by the fact that the various "competitors" are targeting subgroups of patients that are either non-overlapping or minimally overlapping with those targeted by ataluren. However, other companies may develop drugs for the same populations.

Target Price: $24/Share

Assume 60% probability of success for DMD and CF

Our valuation is supported by a DCF model using probability-weighted sales estimates for ataluren in DMD (60%) and CF (60%) modeled through 2030. We use a 38% tax rate and a 12% discount rate, and we arrive at a $30 valuation based on current share count. We conservatively assume that PTC will raise additional capital in 2015 and therefore adjust our valuation by adding 5 to 8M additional shares.

- **Conservative Target Price:** At current share levels, the $24/share target implies an approximately $600M market cap for a Phase III orphan drug company. With 5 to 8M additional shares, the $24 target implies a $700-800M market cap. Even at our target, PTCT is still at a significant discount to its public market comps, most importantly, Sarepta (SRPT) at $1.4B in market cap. Recent IPO Prosensa (RNA) has a market cap of $1.0BM

We assign no value to the SMA program or to the potential for an early approval in Europe (filing under review), both of which could provide upside.

Exhibit 3: Summary of DCF Valuation

DCF valuation		Current	+5M	+8M		Shares	Price	Market cap
Ataluren	558	$22	$19	$17		24.9	$24	$592
Net Cash (Pro forma)	186	$7	$6	$6		29.9	$24	$710
Total	**744**	**$30**	**$25**	**$23**		32.9	$24	$782

Source: Credit Suisse estimates.

The biggest levers in our valuations are

(1) **Probability of Success:** We use 60% for ataluren in DMD and 60% for ataluren in CF. This means that our target price is designed to be achieved ahead of the Phase III

results. We would expect the probability to go higher on positive Phase III results (or potentially based on data from the ongoing extension studies in DMD).

(2) **Pricing of Ataluren:** We assume a gross price of $250,000 per year in the United States and $225,000 in Europe. The pricing is in-line with other orphan drugs but may depend on the magnitude of benefit demonstrated in Phase III.

(3) **Timing of U.S. and EU Approvals**: We assume U.S. and EU launches in 2016 for DMD and 2017 for CF following positive Phase III data in mid-2015 and 2016, respectively. An earlier EU approval for DMD in early 2014 is possible, as PTCT has filed for conditional approval. PTCT may also file for conditional approval in the EU for the CF indication later this year. We assume that a confirmatory Phase III trial is required for both indications and do not assign any value to a potential early approval.

(4) **We Assume that PTCT Enters into a Partnership for the Ex-US/Ex-EU Rights:** While it is possible that PTCT could launch the product globally, we assume direct sales only in United States and EU. We assume a high 30% royalty on all other sales, reflecting a post-Phase III partnership.

Favorable Public Comps

Companies developing drugs for ultra-orphan diseases include several multibillion-dollar companies (e.g., VRTX, ALXN, and BMRN). These companies demonstrate that small biotech companies with an effective targeted therapy for a small patient population can successfully transition to a fully integrated commercial biotech.

Closest Comps are 2.4 to 3.3 Times the Valuation of PTCT: Sarepta (SRPT) and Prosensa (RNA) are developing therapies that target 13% of DMD. This 13% is distinct from the 13% that PTCT is targeting, so they are not directly competitive but are very comparable. Sarepta's and Prosensa's technology may be applicable to an increasing portion of the DMD population, but unlike PTCT, Sarepta and Prosensa do not have a late stage program in any other large market opportunity. PTCT has a planned Phase III in 10% of CF patients. Sarepta has completed a small placebo-controlled Phase II trial in DMD with eteplirsen and has plans to initiate a Phase III trial and Prosensa's drisapersen is in Phase III development.

Exhibit 4: **Public Market Comps for Orphan Drug Companies**

Company	Ticker	Price 7/12/13	Lead Product	Stage	Shares	Cash	Debt	Mark. Cap	Ent. Val
Alexion Pharmaceuticals Inc.	ALXN	$114.26	Soliris	Marketed	195.1	1,023.0	149.0	22,287.0	21,413.0
Vertex Pharmaceuticals	VRTX	$87.79	Kalydeco	Marketed	218.7	1,302.4	739.9	19,195.5	18,633.0
BioMarin Pharmaceutical Inc.	BMRN	$64.81	Naglazyme, etc	Marketed	138.9	401.9	109.8	9,000.4	8,708.3
Isis Pharmaceuticals	ISIS	$33.72	Kynamro	Marketed	102.7	419.4	156.5	3,462.9	3,200.0
Alnylam	ALNY	$50.04	ALN-TTR02	Phase II	62.1	236.0	0.0	3,109.1	2,873.1
Aegerion Pharmaceuticals Inc.	AEGR	$77.41	Lomitapide	Marketed	28.8	140.7	10.3	2,231.0	2,100.5
Sarepta Therapeutics Inc.	SRPT	$44.33	Eteplirsen	Phase II	31.9	167.9	1.7	1,413.6	1,247.5
Synageva BioPharma Corp.	GEVA	$47.15	Sebelipase alfa	Phase III	27.2	311.8	0.0	1,281.9	970.0
Prosensa	RNA	$27.75	Drisapersen	Phase III	35.9	130.4	8.6	996.3	874.4
Raptor Pharmaceutical Corp.	RPTP	$10.77	Procysbi	Marketed	55.4	58.6	25.0	597.0	563.4
PTC Therapeutics	PTCT	$16.94	Ataluren	Phase III	24.9	186.0	3.8	422.5	240.3
Corcept Therapeutics Inc.	CORT	$1.80	Korlym	Marketed	99.8	81.5	32.8	179.7	131.0
Amicus Therapeutics Inc.	FOLD	$2.36	Migalastat HCl	Phase III	49.6	84.8	0.6	117.1	33.0
Average								***4,945.7***	***4,691.4***
Median								***1,413.6***	***1,247.5***

Source: Company data, Credit Suisse estimates

$1B Market Opportunity for Ataluren

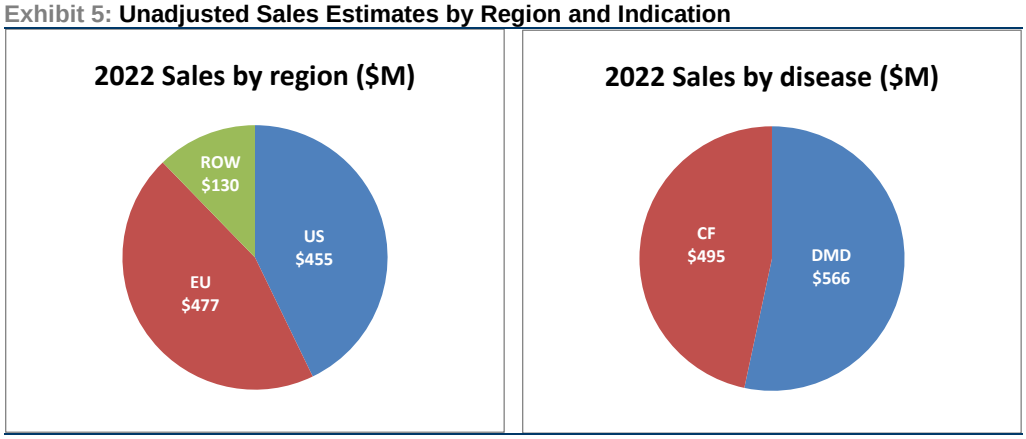
We build our ataluren models for DMD and CF in a similar manner. We take current estimated patient numbers for both diseases, take the percentage of patients believed to have nonsense mutations, apply a high penetration rate, and assume an annual gross price over $200,000/year (US $250,000; EU $225,000).

Markets not currently targeted by competitors

We assume that PTCT retains commercial rights to ataluren in the United States and EU and has a partner selling ROW (30% royalty, after Phase III data).

Our sales assumptions drive 2022 unadjusted global sales estimates of just over $1B, including $455M in the United States and $477M in Europe (probability-adjusted sales of $273M and $286M, respectively). By indication, our unadjusted global sales estimates include $566M in DMD and $495M in CF (probability adjusted sales of $340M and $297M, respectively).

Exhibit 5: Unadjusted Sales Estimates by Region and Indication



Either indication could support upside from current valuation

Source: Credit Suisse estimates.

DMD Modeling Assumptions:

- Ataluren addresses 13% of DMD patients. (See Exhibit 6.)
- Among this 13% of patients, we assume that 80% are eligible for treatment in the United States (a smaller 70% in the EU and 60% in ROW). This assumes that ataluren's label does not limit its use by age or walking ability (as do the entry criteria in Phase III).
- We assume relatively high penetration rates of 70% in the United States, 60% in the EU, and 50% for ROW.
- We use a gross price of $250,000 per year in the United States and $225,000 per year in the EU, with a gross to net adjustment of 13% in the United States and approaching 20% in the EU. Our net price assumption for ROW is the same as the EU.
- We assume an 85% compliance rate.

CF Modeling Assumptions:

- Ataluren addresses 10% of CF patients. (See Exhibit 7.)
- Among this 10% of patients, we assume that 60% are eligible for treatment in the United States (a smaller 55% in the EU and 50% in ROW). We use a lower addressable market in CF than in DMD because of the exclusion of TOBI in the Phase III trial and the potential for other drugs to be used for patients that have a

nonsense mutation on one chromosome and a different addressable mutation on the other.

- We assume relatively high penetration rates of 60% in the United States, 50% in the EU, and 60% for ROW.
- We use the same gross and net price assumptions as DMD.
- We also use a compliance rate of 85%.

Exhibit 6: DMD Sales Build

	2016	2017	2018	2019	2020	2021	2022	2023	2024	2025	2026	2027
DMD patients (US)	13,525	13,796	14,072	14,353	14,640	14,933	15,232	15,536	15,847	16,164	16,487	16,817
Patients with nonsense mutation	1,758	1,793	1,829	1,866	1,903	1,941	1,980	2,020	2,060	2,101	2,143	2,186
% Patients with nonsense mutation	13%	13%	13%	13%	13%	13%	13%	13%	13%	13%	13%	13%
Patients eligible for Ataluren	1,407	1,435	1,463	1,493	1,523	1,553	1,584	1,616	1,648	1,681	1,715	1,749
% Eligible for Ataluren	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%
% penetration	10.0%	50.0%	65.0%	70.0%	70.0%	70.0%	70.0%	70.0%	70.0%	70.0%	70.0%	70.0%
Total patients	141	717	951	1,045	1,066	1,087	1,109	1,131	1,154	1,177	1,200	1,224
Net new patients	141	577	234	94	21	21	22	22	23	23	24	24
Price	$250,000	$250,000	$250,000	$250,000	$250,000	$250,000	$250,000	$250,000	$250,000	$250,000	$250,000	$250,000
Price increases	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%
Gross to net	13%	13%	13%	13%	13%	13%	13%	13%	13%	13%	13%	13%
Net price	$217,500	$217,500	$217,500	$217,500	$217,500	$217,500	$217,500	$217,500	$217,500	$217,500	$217,500	$217,500
Compliance	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%
Annual sales ($M)	**$26**	**$133**	**$176**	**$193**	**$197**	**$201**	**$205**	**$209**	**$213**	**$218**	**$222**	**$226**
DMD patients (EU)	20,808	21,224	21,649	22,082	22,523	22,974	23,433	23,902	24,380	24,867	25,365	25,872
Patients with nonsense mutation	2,705	2,759	2,814	2,871	2,928	2,987	3,046	3,107	3,169	3,233	3,297	3,363
% Patients with nonsense mutation	13%	13%	13%	13%	13%	13%	13%	13%	13%	13%	13%	13%
Patients eligible for Ataluren	1,894	1,931	1,970	2,009	2,050	2,091	2,132	2,175	2,219	2,263	2,308	2,354
% Eligible for Ataluren	70%	70%	70%	70%	70%	70%	70%	70%	70%	70%	70%	70%
% penetration	5.0%	15.0%	30.0%	50.0%	60.0%	60.0%	60.0%	60.0%	60.0%	60.0%	60.0%	60.0%
Total patients	135	414	844	1,435	1,757	1,792	1,828	1,864	1,902	1,940	1,978	2,018
Net new patients	135	279	430	591	322	35	36	37	37	38	39	40
Price	$225,000	$225,000	$225,000	$225,000	$225,000	$225,000	$225,000	$225,000	$225,000	$225,000	$225,000	$225,000
Price increases	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%
Gross to net	12%	15%	17%	19%	20%	20%	20%	20%	20%	20%	20%	20%
Net price	$198,000	$191,250	$186,750	$182,250	$180,000	$180,000	$180,000	$180,000	$180,000	$180,000	$180,000	$180,000
Compliance	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%
Annual sales ($M)	**$23**	**$67**	**$134**	**$222**	**$269**	**$274**	**$280**	**$285**	**$291**	**$297**	**$303**	**$309**
DMD patients (ROW)	7,283	7,428	7,577	7,729	7,883	8,041	8,202	8,366	8,533	8,704	8,878	9,055
Patients with nonsense mutation	947	966	985	1,005	1,025	1,045	1,066	1,088	1,109	1,131	1,154	1,177
% Patients with nonsense mutation	13%	13%	13%	13%	13%	13%	13%	13%	13%	13%	13%	13%
Patients eligible for Ataluren	568	579	591	603	615	627	640	653	666	679	692	706
% Eligible for Ataluren	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%
% penetration	0.0%	10.0%	20.0%	30.0%	40.0%	50.0%	50.0%	50.0%	50.0%	50.0%	50.0%	50.0%
Total patients	0	97	197	301	410	523	533	544	555	566	577	589
Net new patients	0	97	100	104	109	113	10	11	11	11	11	12
Price	$200,000	$200,000	$200,000	$200,000	$200,000	$200,000	$200,000	$200,000	$200,000	$200,000	$200,000	$200,000
Price increases	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%
Gross to net	10%	10%	10%	10%	10%	10%	10%	10%	10%	10%	10%	10%
Net price	$180,000	$180,000	$180,000	$180,000	$180,000	$180,000	$180,000	$180,000	$180,000	$180,000	$180,000	$180,000
Compliance	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%
Annual sales ($M)	**$0**	**$15**	**$30**	**$46**	**$63**	**$80**	**$82**	**$83**	**$85**	**$87**	**$88**	**$90**
EU Roayties	$0	$4	$9	$14	$19	$24	$24	$25	$25	$26	$26	$27

Source: Credit Suisse estimates.

Exhibit 7: Cystic Fibrosis Sales Build

US Cystic Fibrosis	2017	2018	2019	2020	2021	2022	2023	2024	2025	2026	2027
Cystic fibrosis patients	33,959	34,638	35,331	36,037	36,758	37,493	38,243	39,008	39,788	40,584	41,395
% Growth	2.0%	2.0%	2.0%	2.0%	2.0%	2.0%	2.0%	2.0%	2.0%	2.0%	2.0%
CF patients with Class 1 mutations	**3,396**	**3,464**	**3,533**	**3,604**	**3,676**	**3,749**	**3,824**	**3,901**	**3,979**	**4,058**	**4,140**
Nonsense	10%	10%	10%	10%	10%	10%	10%	10%	10%	10%	10%
Eligible CF patients addressible with Ataluren	**2,038**	**2,078**	**2,120**	**2,162**	**2,205**	**2,250**	**2,295**	**2,340**	**2,387**	**2,435**	**2,484**
% Eligible for Ataluren	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%	60%
Patients on Ataluren	**204**	**623**	**1,060**	**1,297**	**1,323**	**1,350**	**1,377**	**1,404**	**1,432**	**1,461**	**1,490**
Penetration rate	10.0%	30.0%	50.0%	60.0%	60.0%	60.0%	60.0%	60.0%	60.0%	60.0%	60.0%
Compliance	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%
Gross Price - Ataluren	$250,000	$250,000	$250,000	$250,000	$250,000	$250,000	$250,000	$250,000	$250,000	$250,000	$250,000
% Increase	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%
Gross to net	13%	13%	13%	13%	13%	13%	13%	13%	13%	13%	13%
Net Price - Ataluren	$217,500	$217,500	$217,500	$217,500	$217,500	$217,500	$217,500	$217,500	$217,500	$217,500	$217,500
Ataluren US sales ($ in MM) - CF	**37.7**	**115.3**	**196.0**	**239.8**	**244.6**	**249.5**	**254.5**	**259.6**	**264.8**	**270.1**	**275.5**

EU Cystic Fibrosis	2017	2018	2019	2020	2021	2022	2023	2024	2025	2026	2027
Cystic fibrosis patients	42,448	43,297	44,163	45,046	45,947	46,866	47,804	48,760	49,735	50,730	51,744
% Growth	2.0%	2.0%	2.0%	2.0%	2.0%	2.0%	2.0%	2.0%	2.0%	2.0%	2.0%
CF patients with Class 1 mutations	**4,245**	**4,330**	**4,416**	**4,505**	**4,595**	**4,687**	**4,780**	**4,876**	**4,973**	**5,073**	**5,174**
Nonsense	10%	10%	10%	10%	10%	10%	10%	10%	10%	10%	10%
Eligible CF patients addressible with Ataluren	**2,335**	**2,381**	**2,429**	**2,478**	**2,527**	**2,578**	**2,629**	**2,682**	**2,735**	**2,790**	**2,846**
% Eligible for Ataluren	55%	55%	55%	55%	55%	55%	55%	55%	55%	55%	55%
Patients on Ataluren	117	357	729	1,239	1,264	1,289	1,315	1,341	1,368	1,395	1,423
Penetration rate	5.0%	15.0%	30.0%	50.0%	50.0%	50.0%	50.0%	50.0%	50.0%	50.0%	50.0%
Compliance	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%
Gross Price - Ataluren	$225,000	$225,000	$225,000	$225,000	$225,000	$225,000	$225,000	$225,000	$225,000	$225,000	$225,000
% Increase	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%
Gross to net	15%	17%	19%	20%	20%	20%	20%	20%	20%	20%	20%
Net Price - Ataluren	$191,250	$186,750	$182,250	$180,000	$180,000	$180,000	$180,000	$180,000	$180,000	$180,000	$180,000
Ataluren EU sales ($ in MM) - CF	**19.0**	**56.7**	**112.9**	**189.5**	**193.3**	**197.2**	**201.1**	**205.2**	**209.3**	**213.4**	**217.7**

ROW launch

ROW Cystic Fibrosis	2017	2018	2019	2020	2021	2022	2023	2024	2025	2026	2027
Cystic fibrosis patients	10,927	11,255	11,593	11,941	12,299	12,668	13,048	13,439	13,842	14,258	14,685
% Growth	3.0%	3.0%	3.0%	3.0%	3.0%	3.0%	3.0%	3.0%	3.0%	3.0%	3.0%
CF patients with Class 1 mutations	**1,093**	**1,126**	**1,159**	**1,194**	**1,230**	**1,267**	**1,305**	**1,344**	**1,384**	**1,426**	**1,469**
Nonsense	10%	10%	10%	10%	10%	10%	10%	10%	10%	10%	10%
Eligible CF patients addressible with Ataluren	**546**	**563**	**580**	**597**	**615**	**633**	**652**	**672**	**692**	**713**	**734**
% Eligible for Ataluren	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%	50%
Patients on Ataluren	0	56	116	179	246	317	391	403	415	428	441
Penetration rate	0.0%	10.0%	20.0%	30.0%	40.0%	50.0%	60.0%	60.0%	60.0%	60.0%	60.0%
Compliance	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%	85.0%
Gross Price - Ataluren	$200,000	$200,000	$200,000	$200,000	$200,000	$200,000	$200,000	$200,000	$200,000	$200,000	$200,000
% Increase	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%
Gross to net	10%	10%	10%	10%	10%	10%	10%	10%	10%	10%	10%
Net Price - Ataluren	$180,000	$180,000	$180,000	$180,000	$180,000	$180,000	$180,000	$180,000	$180,000	$180,000	$180,000
Ataluren ROW sales ($ in MM) - CF	**0.0**	**8.6**	**17.7**	**27.4**	**37.6**	**48.5**	**59.9**	**61.7**	**63.5**	**65.4**	**67.4**
Ataluren ROW royalties ($ in MM) - CF	**0.0**	**2.6**	**5.3**	**8.2**	**11.3**	**14.5**	**18.0**	**18.5**	**19.1**	**19.6**	**20.2**

Source: Company data, Credit Suisse estimates

Targeting 13% of DMD

DMD is a genetic disease caused by a mutation in the gene for dystrophin, a structural protein found in muscles. Lack of dystrophin or the production of nonfunctional dystrophin leads to progressive muscle weakening.

Ataluren is the only drug specifically targeting nonsense mutations

DMD is an X-linked genetic disease found only in boys, and the frequency of the disease is approximately 1 in 3,500 male births. Boys with the disease lose muscle strength and typically become wheelchair bound by the age of 10-15 years. Muscle weakness ultimately leads to respiratory problems and death, with the typical DMD patient living to the age of 25-35.

Current treatments include steroids, which have significant side effects and do not address the fundamental underlying cause of the disease.

Several new agents are in development, and all have the same treatment goal: increase the production of dystrophin by targeting the specific genetic mutation at the root cause of the disease. (See Exhibit 8.) Because there are literally thousands of possible mutations that can lead to DMD, each approach is applicable only to a distinct and usually nonoverlapping subset of patients. This is the case for ataluren, which targets 13% of DMD patients; this 13% is not addressed by other drugs in late stage development.

The mechanism of action for ataluren is discussed in greater detail on page 25.

Exhibit 8: DMD Drugs in Development

Company	Drug	Target	%DMD population	Phase	Largest Trial
PTC Therapeutics	ataluren	Nonsense mutation	13%	Phase III	174
GSK/Prosensa	drisapersen	Exon 51 skipping	13%	Phase III	180
Sarepta	eteplirsen	Exon 51 skipping	13%	Phase IIb	12
GSK/Prosensa	PRO-044	Exon 44 skipping	6%	Phase I/II	18
GSK/Prosensa	PRO-045	Exon 45 skipping	8%	Phase I/II	45
Sarepta	SRP-4045	Exon 45 skipping	8%	Preclinical	NA
GSK/Prosensa	PRO-053	Exon 53 skipping	8%	Preclinical	NA
Sarepta	SRP-4053	Exon 53 skipping	8%	Preclinical	NA
Sarepta	SRP-4050	Exon 50 skipping	5%	Preclinical	NA
GSK/Prosensa	PRO-052	Exon 52 skipping	3%	Preclinical	NA
GSK/Prosensa	PRO-055	Exon 55 skipping	3%	Preclinical	NA

Source: Company data, ClinicalTrials.gov, Credit Suisse estimates.

DMD Phase III Trial Follows Significant Phase II Experience

PTCT is conducting a randomized placebo-controlled Phase III trial of ataluren in ~220 boys with DMD who are seven years or older. The primary endpoint of the trial is change in six-minute walking distance (6MWD) from baseline to 52 weeks. (See Exhibit 9.)

Phase III trial is optimized for success

- **6MWD is an FDA-validated endpoint for a variety of diseases** including respiratory diseases and genetic diseases effecting muscle strength. 6MWD has never been used as an endpoint in a Phase III trial for DMD, but it is currently the standard being used to evaluate all late stage drugs in DMD.

Patients need to meet specific walking requirements at baseline in order to qualify for the Phase III study. While ataluren is anticipated to have clinical benefit to patients regardless of age and walking ability, the baseline characteristics in the Phase III trial were chosen

because of the limitations of the endpoint and the need to demonstrate a statistically significant result in a 12-month study.

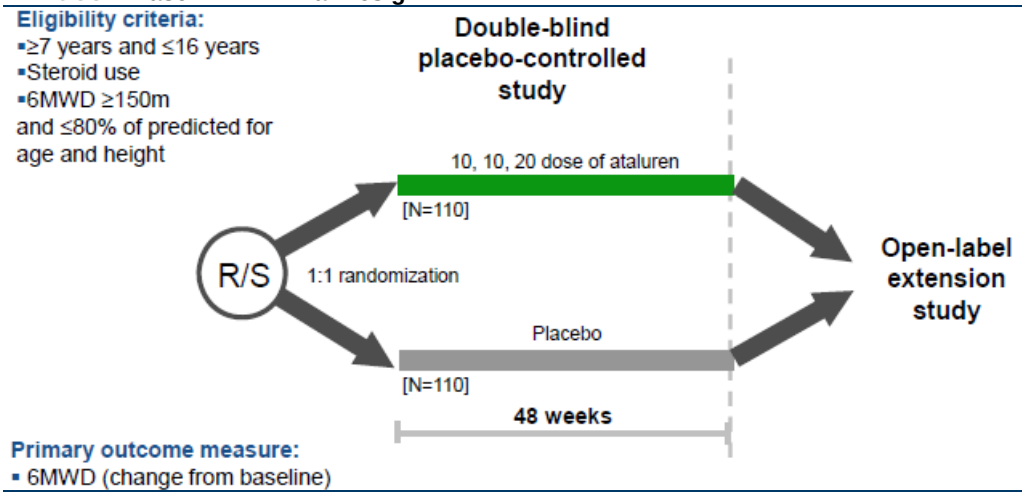
- **The baseline walking criteria and the age range were selected** based on the results seen among patients treated with placebo in PTCT's large Phase II trial.

Ataluren is given orally three times per day at a dose of 10 mg, 10 mg, and 20 mg. This dose was selected based on results from the large placebo-controlled Phase II trial.

- **In the Phase II the 10, 10, 20 mg dose showed a strong nonstatistically significant trend** (p value 0.056), which was highly statistically significant when adjusted for the proposed Phase III enrollment criteria (p value 0.0096).

Exhibit 9: Phase III DMD Trial Design

Simple design with better patient selection



Source: Company data

Exhibit 10: Phase III Trial Closely Mirrors Phase II with Tighter Entry Criteria

Twice as many patients per arm

	Phase II	Phase III
# of patients	174	240
Design	Placebo controlled	Placebo controlled
Stratification	Age, corticosteroid use, baseline 6MWD	Age, time on steroids, baseline 6MWD
Dose(s)	10,10,20 mg 20,20,40 mg placebo	10,10,20 mg placebo
Primary endpoint	6MWD	6MWD
Duration	48 weeks	48 weeks
Age	≥5	7-16
Baseline 6MWD	≥75m	≥150m and ≤80% of predicted
	No requirement for steroid use	Steroid use for at least 6 months
Status	Completed	Enrollment ongoing Data in H1:15

Source: Company data, Credit Suisse estimates.

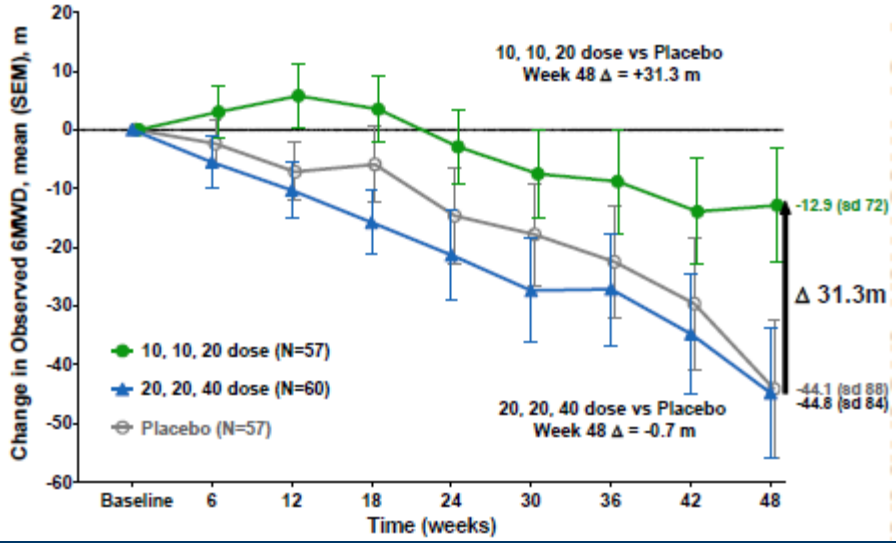
Phase II Data Provide Strong Evidence to Predict Success in Phase III

PTCT ran one of the largest placebo-controlled trials in DMD to date. The study included 174 patients, ages five and older, randomized to one of two doses of ataluren or placebo for one year. On an intent-to-treat basis, neither treatment arm reached statistical significance, but there are several factors that make us confident that the trial demonstrated clear efficacy of ataluren.

1. **Very Strong Trend:** The low dose group showed a 31.3m difference versus placebo with a p value of 0.0561. (See Exhibit 11.) While this is not a statistically significant result, it does imply 94% confidence in the result.
2. **The Same Dose Showed Meaningful Clinical Activity in Cystic Fibrosis:** (See the section "Targeting 10% of Cystic Fibrosis" later in this report.)
3. **The Result for the 10, 10, 20 mg Dose Group Is Statistically Significant and More Robust When Adjusted for the Population Being Tested in the Phase III:** In this analysis, the 10, 10, 20 dose group showed a 49.9m difference versus placebo with a p value of 0.0096. (See Exhibit 12.) The modified inclusion/exclusion criteria in Phase III were rationally designed based on the natural history of the disease as demonstrated by the large placebo group in the Phase II. Importantly, other drug developers are following in PTCT's footsteps and modeling their Phase III entry criteria along the same parameters.

Exhibit 11: 10, 10, 20 Dose Demonstrates Meaningful Improvement over Placebo

10,10, 20 p value = 0.0561

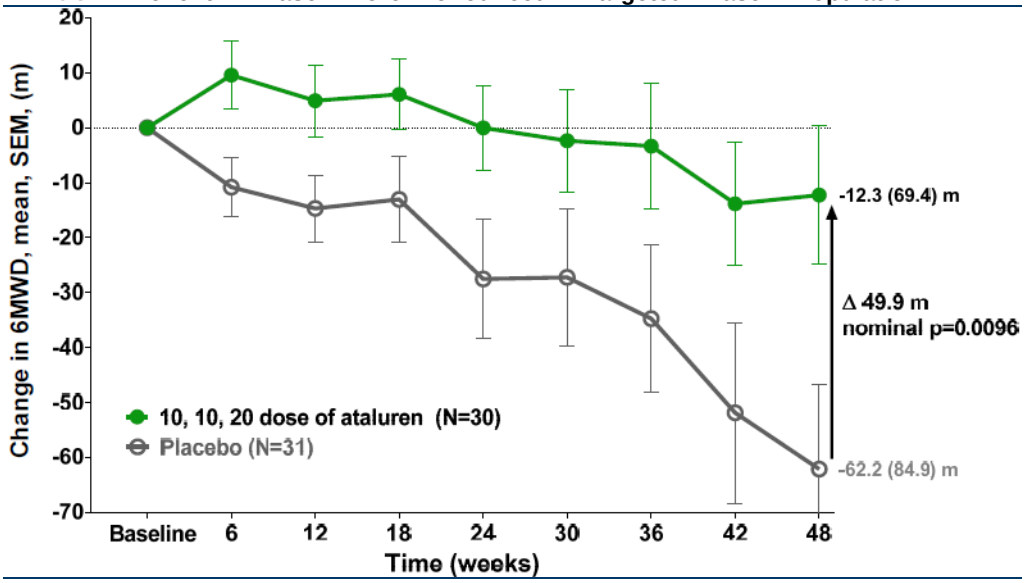


31.1M is clinically meaningful

Source: Company data

Exhibit 12: Benefit in Phase II More Pronounced in Targeted Phase III Population

Adjusted analysis provides compelling benefit



Source: Company data

Natural History of DMD Points the Way to Phase III

Rational changes driven by real experience in Phase II

One of the key take-aways from the Phase II trial was that placebo patients have highly variable changes in 6MWD over the course of a one-year study, depending on age and baseline walking ability. This is depicted in Exhibit 13, which shows the one-year change in 6MWD for each of the placebo-treated patients in the Phase II.

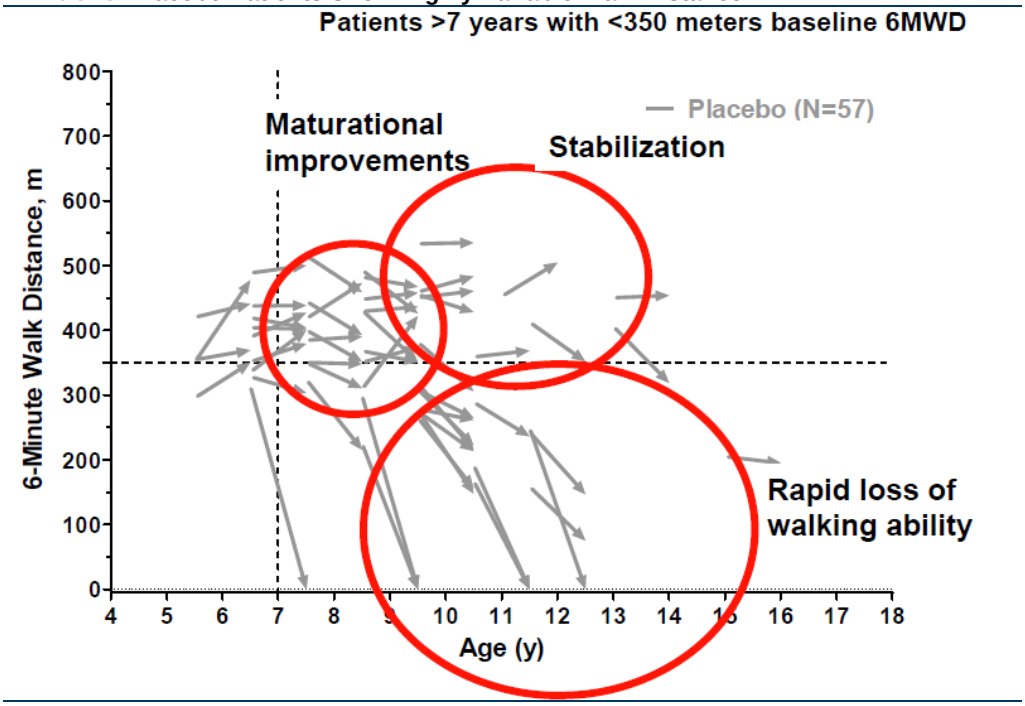
- **Patients younger than seven** typically improve their walking ability over the course of a year. This is because these children are growing and maturing faster than their disease is progressing.
- **High-functioning patients with baseline 6MWD above 350m** tend not to progress over the course of one year. These patients will eventually progress, but measuring a clinical benefit in these patients in a one-year study is not feasible using 6MWD.
- **Patients older than seven with 6MWD below 350m at baseline** are the subset of patients most likely to deteriorate over the course of one year. These patients provide a testable subgroup in which to measure the impact of a disease-modifying drug in a one-year period.

The Phase III trial will test ataluren only in patients older than seven with a baseline 6MWD below 350m. This population showed a 49.9m difference between the treated group and the placebo group in the Phase II trial with a p value of 0.0096. (See Exhibit 13.) This large subgroup included 61 patients (30 on ataluren, 31 on placebo).

Other companies developing drugs for DMD have used this same data set to design their trials. For example, Sarepta's Phase II trial in DMD patients required patients to be between 7 and 13 years of age and to have a 6MWD of 200-400m at baseline. The use of these entry criteria by other companies supports the conclusions from PTCT.

Exhibit 13: Placebo Patients Show Highly Variable Walk Distance

Goal is to exclude patients with stable 6 MWD



Source: Company data

Controversy #1: Bell-Shaped Dose Response Curve

Sometimes more is not better

The high dose ataluren arm in the Phase II DMD trial demonstrated absolutely no benefit over placebo. This observation has raised some concerns regarding the mechanism of ataluren, as most drugs tend to show increased activity with increasing doses. However, many biologic responses show bell-shaped dose response curves, and that appears to be the case with ataluren.

We believe that:

1. The Phase II trial clearly identified an active dose, although we concede that the trial failed to convincingly demonstrate an optimal dose.
2. The available preclinical data support the findings that higher doses of ataluren can reverse the activity seen at lower doses.

Preclinical Observations

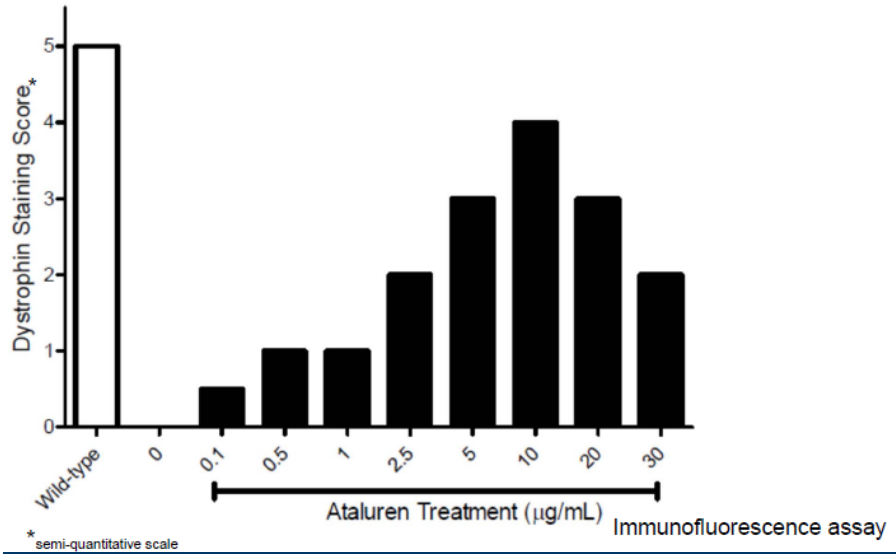
Ataluren has demonstrated a “bell-shaped” dose response across a number of preclinical and in vitro assays, paralleling the clinical findings:

- Dystrophin expression in mdx mouse myotubes (Exhibit 14)
- GAGs in Hurler mouse model expression (Exhibit 15)

The bell-shaped dose response is believed to be due to the presence of two binding sites for ataluren on the ribosome: a high-affinity site and low-affinity site. The high-affinity site is bound first by ataluren at lower concentrations, and when ataluren binds this site, it is believed to promote the read-through activity. At higher concentrations, ataluren binds to the low-affinity site and antagonizes the activity from the ataluren bound to the high-affinity site.

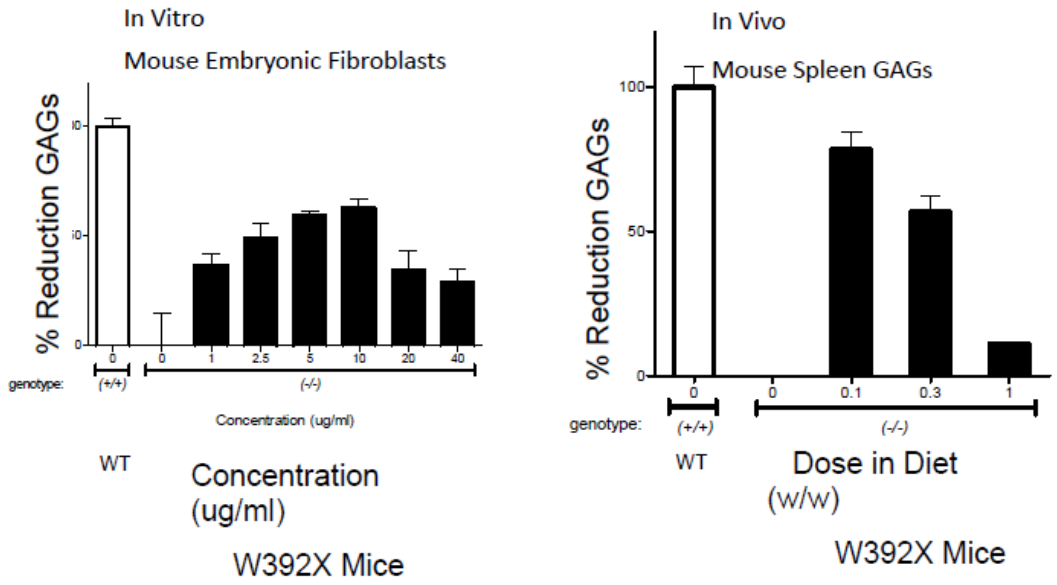
Exhibit 14: Dystrophin Expression Bell-Shaped Dose-Response in Mdx Mouse Myotubes

Evidence of high dose effect in animal models



Source: Company data

Exhibit 15: Bell-Shaped Dose Curve Observed with In Vitro and In Vivo Experiment in Hurler Mouse Model



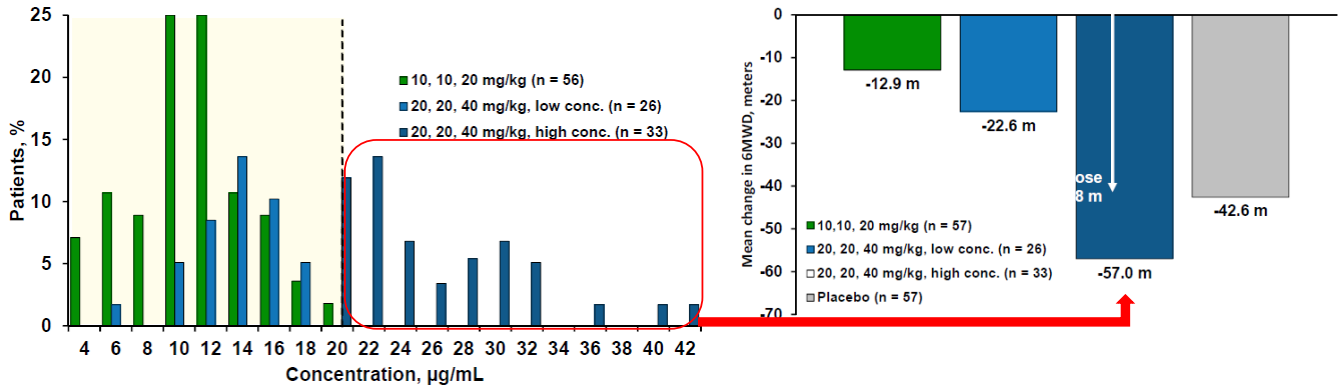
Source: Company data

Clinical Observations

For us, the most convincing dose relationship data come from the analysis of plasma drug levels in the Phase II trial.

PTCT examined two different doses in the DMD Phase II study: a low dose (10, 10, 20 mg/kg) and a high dose (20, 20, 40 mg/kg). Patients in the Phase II trial who were treated at the high dose had a wide range of drug levels in the body. Patients with the highest blood levels had no measurable response, and patients with lower levels (similar to the low dose) had clinical responses in-line with the lower dose. (See Exhibit 16.) This supports the preclinical work that ataluren demonstrates a bell-shaped dose curve.

Exhibit 16: 6MWD Were Better in Patients with Low Plasma Levels in the 20, 20, 40 mg/kg Cohort



Source: Company data

DMD Strategy for Conditional Approval in Europe

Upside to our model if EMA rules favorably

In October 2012, PTC filed for accelerated approval in Europe to treat nmDMD patients based on the randomized Phase II results. PTCT has received its 120-day questions from EMA and expects to provide answers in July 2013. A CHMP recommendation could potentially come in late 2013.

The filing is based on the post-hoc analysis, which analyzes patients based on age and baseline walking criteria. In this analysis, the results of the low dose arm are statistically and clinically significant.

We view this strategy as a long shot and currently assume no value for an early approval in our model. Disclosures in the S-1 provide some indication that EMA may have a relatively negative view of the current filing, but many of their issues may be addressable with further explanation from PTCT, which is the purpose of the 120-day questions.

However, there are several factors that increase the probability of a favorable decision.

- **High Unmet Medical Need:** There are no available drugs for these patients that target the root cause of DMD. Because the disease is progressive, early approval could provide significant clinical benefit for patients.
- **Targeted Population:** Ataluren specifically addresses a class of mutations that are readily identifiable. The ability to target the drug to the correct patient subset provides an extra level of safety margin.
- Patients were stratified at baseline based on age and 6MWD, which means that the subgroups are likely well balanced for other factors that could influence disease progression and treatment response.
- The subgroup of patients in the post-hoc analysis is significant (61 patients), and the safety database is much larger.
- The safety profile of ataluren is very clean, which improves the overall risk benefit.

Clean Safety Profile for Ataluren

Ataluren has been tested in approximately 590 patients, including randomized trials in DMD (Phase II) and CF (Phase III). The large body of data provides ample evidence of clinical activity and a well-defined safety profile.

Data from the DMD trial that tested two doses of ataluren show no clear dose-related side effects for ataluren. The safety of the drug is being further evaluated in long-term extension studies in both the United States and the EU, which could provide additional data in 2014.

Exhibit 17: Data from Ataluren Randomized DMD Phase II Trial

Adverse Event	Placebo N=57	Low Dose N=57	High Dose N=60
Vomiting	39%	56%	45%
Headache	25%	39%	25%
Diarrhea	25%	19%	28%
Nasopharyngitis	23%	23%	17%
Pyrexia	21%	25%	12%
Cough	19%	16%	22%
Abdominal pain upper	16%	16%	22%
URT infection	18%	16%	18%
Fall	12%	19%	10%
Nausea	12%	14%	17%
Abdominal pain	7%	12%	17%
Procedural pain	12%	10%	13%
Pain in extremity	11%	12%	13%
Back pain	9%	16%	10%
Influenza	14%	11%	12%

Source: Company data

Ongoing Extension Studies Could Provide Incremental Safety and Efficacy Data

Most patients opt to stay on drug

There are two ongoing long-term extension studies of ataluren in DMD patients: Study 016 in the United States and Study 019 in Europe. These trials have a somewhat unusual history, but over the course of the next 12-18 months, we expect that the trials will generate meaningful safety and efficacy results.

A Bumpy History

The 174-patient Phase II trial in DMD initially included a voluntary cross-over to a long-term extension study. Patients in all treatment groups were allowed to continue treatment at the high dose of ataluren. Participation in the extension portion was very high, as this was a major incentive for patients to enroll in a placebo-controlled trial. At the time, PTCT was responsible for the conduct of that trial in the United States (Study 016), and its partner Genzyme was responsible for the trial in Europe (Study 019).

When the results of the trial were disclosed and it was discovered that the high dose was not effective, patients in both extension trials were taken off drug. On average, patients had been on the extension approximately 12 weeks, with some on drug for as long as 24 weeks.

- **Study 016:** The U.S. extension study was restarted in spring 2011 following an approximate one-year period of no treatment. Patients were enrolled at the lower dose (10, 10, 20 mg). A total of 107 patients are enrolled in this study, which is now ongoing for two years. The trial was reinitiated solely as a safety study, and no baseline efficacy data were collected. However, the large number of patients on treatment for so long is a positive signal that the drug's risk/benefit profile is favorable.

- **Study 019:** The EU extension trial took much longer to reinitiate, primarily because its partner Genzyme gave back the rights to the drug to PTCT. The trial was eventually reopened in fall 2012, but unlike the US study, the EU study included baseline measurements of function and assessment of efficacy on multiple measures throughout the treatment period. There are now 75 of 96 patients enrolled in the study.

What Will These Trials Tell Us?

We expect that PTCT will provide some update (safety and/or efficacy) from these studies in 2014. The EU trial (Study 019) is likely to have the most robust data, which will include changes from baseline in the following:

- 6MWD,
- Parent/caregiver reported activities of daily living using the EK scale,
- Time function tests,
- Pulmonary function,
- Physical function using the North Star Ambulatory Assessment, and
- Patient and/or parent/caregiver reports of disease status.

These results will not be placebo controlled, but they can be compared to prior baselines and to treatment results from the prior Phase II trial.

The extension trial is also important in that patients will be enrolled that might not qualify for the current Phase III. Specifically, patients who became nonambulatory since the first Phase II trial would still be eligible, and measurement of efficacy other than walking ability will be measured. It is possible that FDA and EMA will use these data to help justify a broader label for all nmDMD patients.

Targeting 10% of Cystic Fibrosis

Ataluren is the only drug targeting nonsense mutations

Cystic fibrosis is a genetic disease affecting approximately 70,000 patients worldwide, in which patients lack or have severely impaired function of the CFTR ion channel. While the disease manifests in many different organs, the primary site of disease is the lungs, where the lack of CFTR activity leads to the production of thick mucus, chronic infection and inflammation, and ultimately destruction of the lungs, requiring transplant. Patients are diagnosed as infants and typically die in their twenties.

Like DMD, there are several drugs in development that treat the root cause of the disease and lead to the production, transport, or activation of functional or partially functional CFTR. (See Exhibit 18.) A small change in the activity of CFTR can have dramatic and potentially disease-modifying effects.

Like DMD, the drugs in development for CF now target specific genetic mutations responsible for the lack of functional CFTR.

- **Kalydeco:** The best example is Vertex's Kalydeco, which is approved specifically for the 4% of patients with G551D mutations. These patients produce a defective CFTR, and Kalydeco increases its activity. Ongoing studies aim to expand that indication to the small percent of patients with other "gating" or partial-function mutations (~1-5%).
- **VX-809 and VX-661:** More recently, VRTX has reported positive results for the combination of VX-809 (or VX-661) in combination with Kalydeco for the more common F508del mutation. This is the most common mutation in CF, found in approximately 70% of patients (~45% of patients have two copies [homozygous]). F508del mutants produce a CFTR that is not properly transported to the cell surface. VX-809 or VX-661 increases the transport of the CFTR to the cell surface, and Kalydeco helps to activate it, leading to a partial restoration of function.
- **Ataluren:** Approximately 10% of CF patients have a nonsense mutation. Patients with a nonsense mutation have a severe form of CF because of the lack of CFTR. These mutations are not addressable with either VX-809 or Kalydeco. Ataluren causes read-through of these inappropriate "stop" mutations and allows the production of full-length CFTR.

Unlike DMD, CF is caused by the loss of a gene that is found on both chromosomes; therefore, a patient must have two mutated genes. For this reason, patients with a nonsense mutation on one chromosome most likely harbor a different type of mutation on the second chromosome (typically F508del). For this reason, there may be more overlap between these drug classes than in DMD, in which each patient has only one mutated gene (only boys and only on the X chromosome).

Exhibit 18: Drugs Targeting the Root Cause of CF

Company	Drug	Stage	Target pts	% of CF
Vertex	Kalydeco	Marketed	G551D	4%
Vertex	Kalydeco/VX809	Phase III	F508del homozygous	45%
Vertex	Kalydeco	Phase III	R117H	3%
Vertex	Kalydeco	Phase III	Other gating mutations	3%
PTC	Ataluren	Phase III	Nonsense mutations	10%
Vertex	Kalydeco/VX661	Phase II	F508del homozygous	45%

Source: Company data, ClinicalTrials.gov, and Credit Suisse estimates.

CF Phase III Builds on Experience from Prior Failed Phase III Trial

PTCT is planning to initiate a confirmatory Phase III trial of ataluren in patients with nonsense mutation cystic fibrosis starting in H1:14. The final design is pending further discussions with regulators, but the current plan is to randomize patients to either the 10, 10, 20 mg dose of ataluren or placebo for 48 weeks. The primary endpoint will be FEV1, and patients will not be enrolled if they are currently on tobramycin (TOBI). Other trial design features including age, baseline characteristics, and stratifications are likely to be similar. (See Exhibit 19.)

With TOBI use excluded, we expect that ataluren can demonstrate at least a 5% improvement in FEV1 versus placebo, and this result should be highly statistically significant.

Exhibit 19: Comparison of Likely Trial Design in Confirmatory Phase III

	1st Phase III	Confirmatory Phase III
# of patients	238	Similar size
Design	Placebo controlled	Placebo controlled
Stratification	Age (<18 vs. ≥18) Inhaled antibiotics (Y/N) Baseline FEV1 (<65% vs. ≥65%)	Likely the same
Dose(s)	10,10,20 mg placebo	10,10,20 mg placebo
Primary endpoint	FEV1	FEV1
Duration	48 weeks	48 weeks
Age	6 years and older	Likely the same
Baseline FEV1	FEV1 >40% and ≤90% of predicted	Likely the same
Other		No use of TOBI

Source: Company data, ClinicalTrials.gov, Credit Suisse estimates.

Confirmatory trial likely very similar to first trial

Prior Phase III Trial Missed on the Primary Endpoint but Showed a Clear Path Forward

On an intent-to-treat basis, the original Phase III trial of ataluren in nonsense mutation CF patients missed its primary endpoint. Compared to placebo, ataluren showed a very modest 3% improvement in FEV1 at week 48 with a p value of 0.124. (See Exhibit 20, left panel.) The curves separated early and stayed separated by an average of 2.3% over the course of the study with a p value of 0.0478.

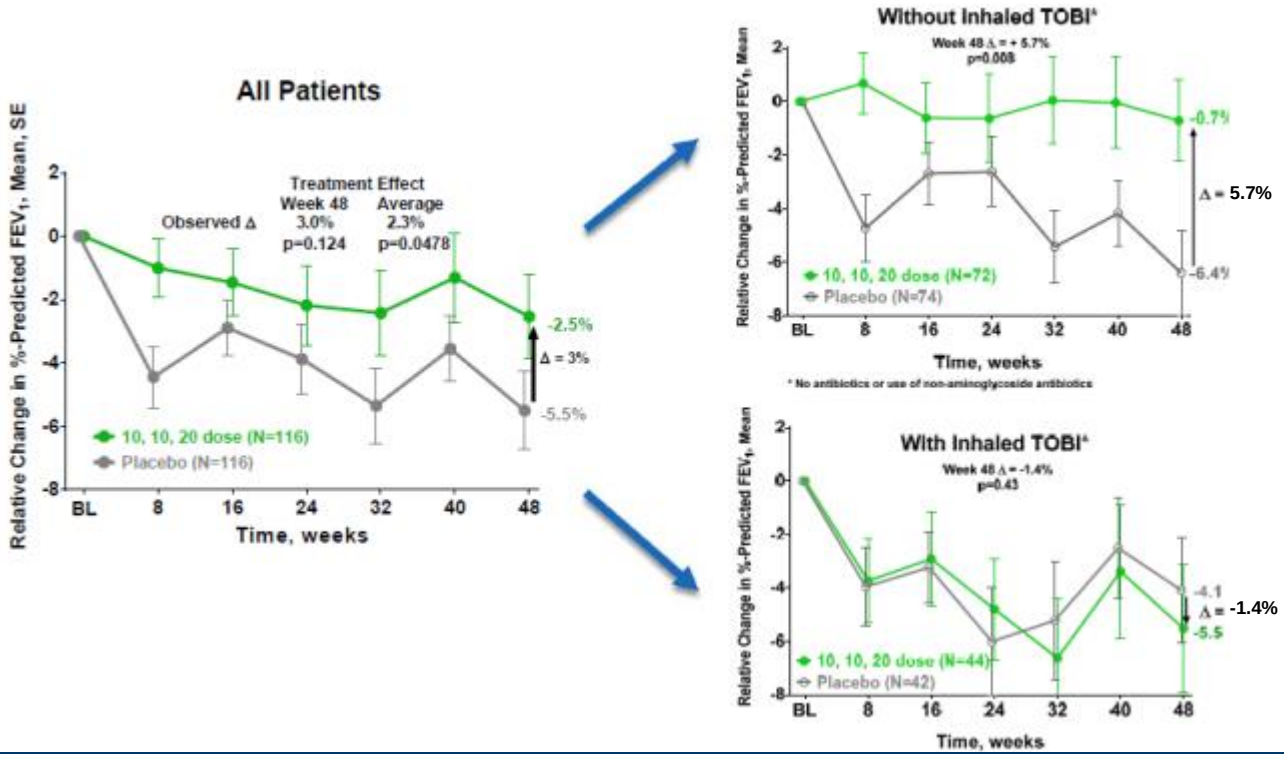
Use of Tobramycin (TOBI) Is Now Contraindicated

The Phase III trial clearly showed that patients receiving TOBI, an FDA-approved inhaled antibiotic, demonstrated no benefit over placebo either at 48 weeks or throughout the study period. (See Exhibit 20, lower right panel.)

Conversely, when patients on TOBI were excluded, the result was much more robust, both in terms of FEV1 improvement (5.7%) and p value (0.008). (See Exhibit 20, top right panel.)

Because the study was very large (232 patients), the subgroups with TOBI (86 patients) and without TOBI (146 patients) were also very large, making the conclusions more statistically believable.

Exhibit 20: Use of TOBI Negates the Positive Effect of Ataluren



Source: Company data

Controversy #2: Interaction with TOBI

In retrospect, the negative interaction with TOBI may have been predicted and certainly should have been tested (at least in animal models) prior to starting a pivotal trial. Many investors view this analysis as data dredging and see the intent-to-treat result as unconvincing.

Our view is that the subgroup analysis with and without TOBI is both valid and convincing for the following reasons:

- Patients were stratified for antibiotic use at baseline. While the stratification was not specifically for TOBI use, it does increase the probability that the ataluren and placebo groups in this subset were well balanced, and in fact, there were nearly identical numbers of patients in each group (72 and 74).
- The majority of patients in the trial were included in the non-TOBI subset. Of the 232 patients in the primary analysis, 146 were included in the subset analysis.
- Subsequent preclinical data strongly support the negative interaction of ataluren and aminoglycoside antibiotics (such as TOBI).

CF Strategy for Conditional Approval in Europe

PTCT is planning to file for conditional approval in nmCF in Europe based on the post-hoc analysis of the completed Phase III trial. Our expectation is that PTCT could file in Q4:13, likely after it completes regulatory discussions regarding the planned Phase III trial. This is a necessary step, as the EMA will typically not consider a drug for conditional approval

unless it believes that a confirmatory Phase III trial will be fully or nearly fully enrolled at the time of approval.

While we view this strategy as a long shot (no value given in our model), there are several aspects of the data that provide support to a conditional approval.

- **High Unmet Medical Need:** Only recently have drugs been approved that target the root cause of CF. The available drugs are not appropriate for the patient population targeted by ataluren.
- **Targeted Population:** Ataluren specifically addresses a class of mutations that are readily identifiable. The ability to target the drug to the correct patient subset provides an extra level of safety margin.
- Patients were stratified at baseline based on antibiotic usage, which means that the subgroups are likely well balanced for other factors that could influence disease progression and treatment response.
- The subgroup of patients who did not use TOBI was large (146 patients), making the subgroup results robust.
- The safety profile of ataluren is very clean, which improves the overall risk benefit.

It Looks Like There's No Stopping PTC: How Ataluren Works

In genetic diseases, there are several types of genetic mutations that can lead to loss of function and ultimately to disease. In recent years, several approaches have been developed that specifically target certain mutations, with dramatic positive results. These approaches include drugs that target the mutated protein and drugs that target the defected genes themselves through interaction with RNA. Some examples include:

Drugs that bind to defective proteins:

- **Activators:** Vertex's Kalydeco for CF gating mutations (~5% of CF) binds to the defective protein in CF and activates it. Kalydeco only works if the protein is made and properly located on the cell surface. That is why the drug initially addresses a small patient population, but it can be combined with other drugs such as VX-809 and potentially ataluren that increase the amount of CFTR that gets to the cell surface.
- **Potentiators:** VRTX's VX809 and VX661 for F508del mutations (45% of CF) bind the defective CFTR and help it "traffic" to the correct location in the cell. Some mutated proteins are not functional because they do not reach the proper location in the cell (e.g., CFTR needs to get to cell surface). These drugs are only marginally active alone but have significantly increased activity in combination with Kalydeco.

Drugs that promote the production of protein from defective genes:

- **Exon Skipping:** SRPT's eteplirsen and RNA's drisapersen edit out the mutated portion of gene, producing an altered but still functional protein. Mutations can shift the reading frame of a gene and produce a nonfunctional and truncated protein. If these mutations fall into certain regions of the gene, they can be overcome by drugs that skip the mutated portion of the gene, leading to the production of a modified version of the native protein, which in certain cases retains enough of the native protein to restore function.
- **Read-through:** PTCT's ataluren binds to the ribosome, allowing full-length proteins to be made from mutated genes. Nonsense mutations introduce an inappropriate "stop" signal into the gene and cause protein production to terminate prematurely. The result is an inactive, unstable, and truncated protein. Ataluren can promote read-through of these stop signals in multiple genetic disease. This mechanism has the potential advantage of targeting a specific type of mutation and not a specific gene or protein, so it may be universally applicable across genetic diseases.

Ataluren Promotes Read-through of Nonsense Mutations

A new drug class for genetic diseases

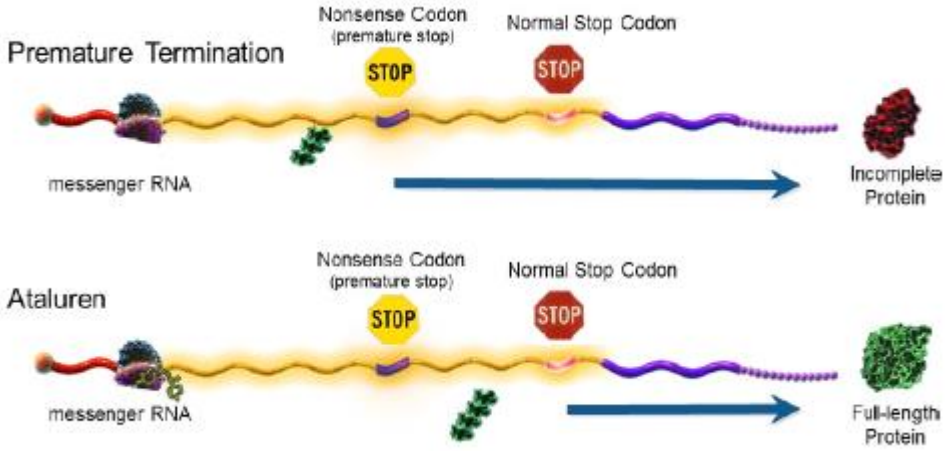
Nonsense mutations are a type of genetic mutation that causes a premature "stop codon" to be introduced into the transcript. When the cellular machinery that normally makes proteins encounters the stop codon, protein production is stopped, and the result is a truncated, unstable, or ineffective protein that leads to disease. Nonsense mutations are found among nearly all genetic diseases for which loss of a protein is the cause. Approximately 10% of these diseases are thought to be due to nonsense mutations.

Ataluren works by directly acting on the ribosome to promote read-through of the stop codon. (See Exhibit 21.) This allows for enough production of functional protein to provide amelioration of the symptoms of the disease.

Importantly, ataluren does not promote read-through of normal stop codons used to terminate the production of the normal, full-length protein. PTCT has studied this extensively in multiple in vitro and in vivo systems, and if it did promote read-through of

normal stop codons, it would have potentially led to multiple side effects in humans, which have not been seen.

Exhibit 21: Diagram of Ataluren Function in Presence of Nonsense Mutation



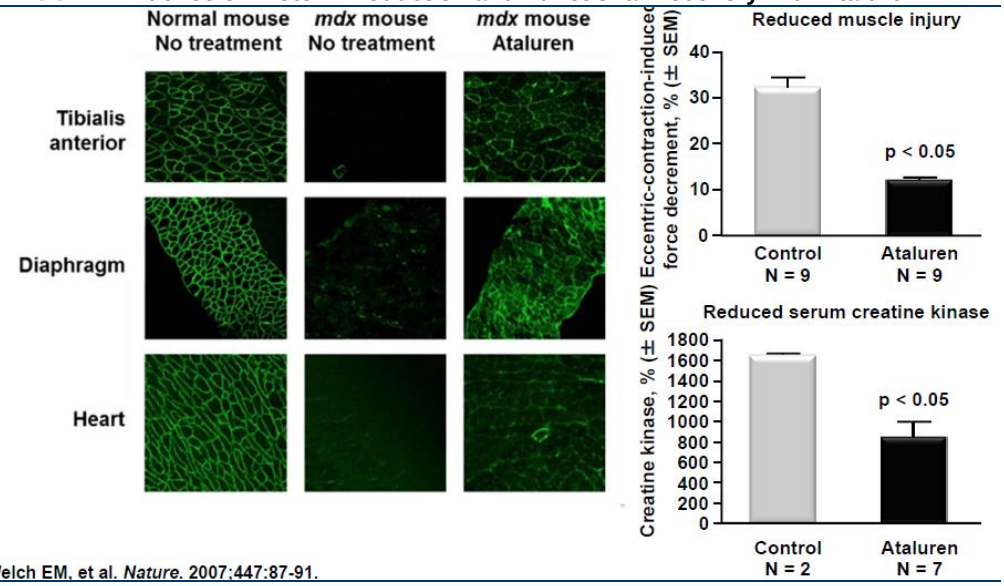
Source: Company data

Production of Functional Protein in Disease Models

Ataluren has demonstrated activity in numerous animal models for nonsense-mediated disease, such as DMD, CF, and MPS.

The activity of ataluren can be measured both in terms of full-length protein produced and restoration of function. In the MDX mouse model of DMD, mice do not make functional dystrophin, and this can be reversed with ataluren treatment. (See Exhibit 22, left panel.) In these same mice, ataluren reduces muscle injury, which is evidence that the dystrophin is functional. (See Exhibit 22, right panel.)

Exhibit 22: Evidence of Protein Production and Functional Recovery with Ataluren



Welch EM, et al. *Nature*. 2007;447:87-91.

Source: Company data

Controversy #3: Not All Agree on Ataluren's Mechanism

The evidence falls strongly in favor of ataluren

A recent publication by McElroy *et al* in the journal *PLoS* called into question the mechanism of action of ataluren. In that author's hands, ataluren (from a source other than PTCT) was not effective in several in vitro assays designed to measure nonsense mutation read-through. The paper also cites prior publications that also questioned the activity of ataluren. The timing of the *PLoS* article (immediately after the IPO) caused heightened investor concerns.

We have had an opportunity to review the recent *PLoS* publication and past literature. We have also had discussions with Dr. McElroy and with members of the scientific team at PTCT, and we have come to the following conclusions regarding the *PLoS* paper and the totality of the data:

- **The Measurement of the In Vitro Activity of Ataluren Is Highly Dependent on the Genetic Construct and the Reporter Assay Used:** Given that McElroy was not able to make ataluren work in any of his models, it is unclear whether his assays were appropriate or not for measuring ataluren's activity (these models are highly artificial and may be subject to many variables that affect the results). A better comparison would be to compare his assays directly with assays where ataluren has worked.
- **Shortcomings of negative studies:** There are six reports of negative in vitro results for ataluren. (1) None tried to replicate the positive studies published. (2) All used G418 as a positive control which is more potent than ataluren and thus the assays may not have been sensitive enough. (3) Five of six used reporter constructs that lacked introns and are therefore less likely to replicate the "natural" setting.
- **Ataluren Is Also a Difficult Molecule to Work With Because of Its Low Solubility:** This can affect results in various settings.
- **Ataluren screening:** PTC identified ataluren using two separate in vitro reporter systems confirmed by tests of activity in more relevant assays: (1) cultured cells from DMD mice, (2) tissue expression in mice, and (3) cultured cells from DMD patients.
- **Activity seen in multiple disease models and patient samples:** PTC and independent investigators have shown activity in (1) DMD, (2) CF, (3) Myoshi myopathy, (4) Usher syndrome, (5) CPT1A deficiency, (6) Batten disease, (7) MPS VI, (8) MMA, (9) Ataxia telangiectasia, and (10) PXE.
- **Clinical Efficacy:** Ataluren has been tested in two large, well-controlled clinical trials in DMD and CF, and both trials showed improvements that are consistent with the proposed mechanism of action. These results trump the results in any in vitro system.
- **Correlation between Clinical and Preclinical Observations:** The negative interaction with certain antibiotics (TOBI) and the bell-shaped dose-response curve were observations from the clinic that were replicated in cell-based assays. This provides one more level of comfort linking the assays to real-world outcomes.

While we believe that this controversy will likely persist and that additional publications could follow, we are confident in the totality of the data supporting ataluren and its proposed mechanism of action.

SMA Program Could Become a Driver in 2014/2015

PTCT has partnered with Roche to develop drugs for the treatment of spinal muscular atrophy. This neurodegenerative disease affects approximately 10,000 children in the United States and is the leading genetic cause of infant death.

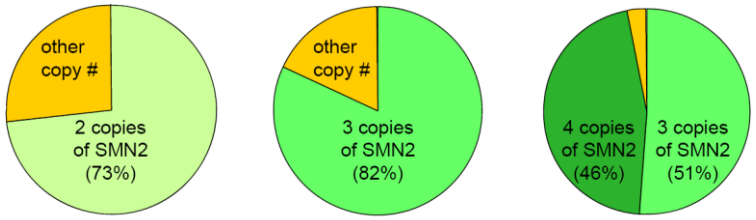
The disease is caused by an inadequate production of a protein called SMN, which is necessary for motor neuron survival. This protein can be produced by one of two genes.

- SMN1 gene is the primary source of the SMN protein. Mutations in the SMN1 gene lead to the development of SMA, as the other gene SMN2 is not sufficient.
- SMN2 gene can also produce functional SMN protein, but the message it encodes is more frequently spliced into an alternative form (70-90% of the time), which codes for a protein that is rapidly degraded and therefore inadequate. (See Exhibit 23.)

The goal of many therapies for SMA is to increase the product from the SMN2 gene to compensate for the loss of SMN1. Human data clearly show that the severity of disease in SMA patients correlates with the copy number of the SMN2 gene. (See Exhibit 23.) The most severe form (Type I) is associated with lower copy number of SMN2 (bottom panel), and the least severe form (Type III) is associated with higher copy number of SMN2. This forms the primary rationale for trying to increase SMN2 activity as a therapeutic intervention.

Exhibit 23: More SMN2 Is Associated with Less Severe SMA (Rationale for Upregulating SMN2)

Type	Age of onset	Life expectancy	Highest function	SMN2 gene copy #[1]	SMN protein % of carrier[2]
I	0 - 3 months	< 2 years	Unable to sit, respiratory insufficiency	~2	~30-40%
II	6 -18 months	> 2 years	Able to sit, cannot stand or walk unaided	~3	~50-60%
III	After 2 years	Adult	Able to sit, stand and walk with restrictions	~ 3 to 4	~60-80%



Charts - courtesy of SMA Foundation

[1] The SMN2 copy number does not always predict SMA type.

[2] SMN protein measured using Western blot in SMA patient fibroblasts (PTC data)

Source: Company data

PTCT Targets Increased SMN2

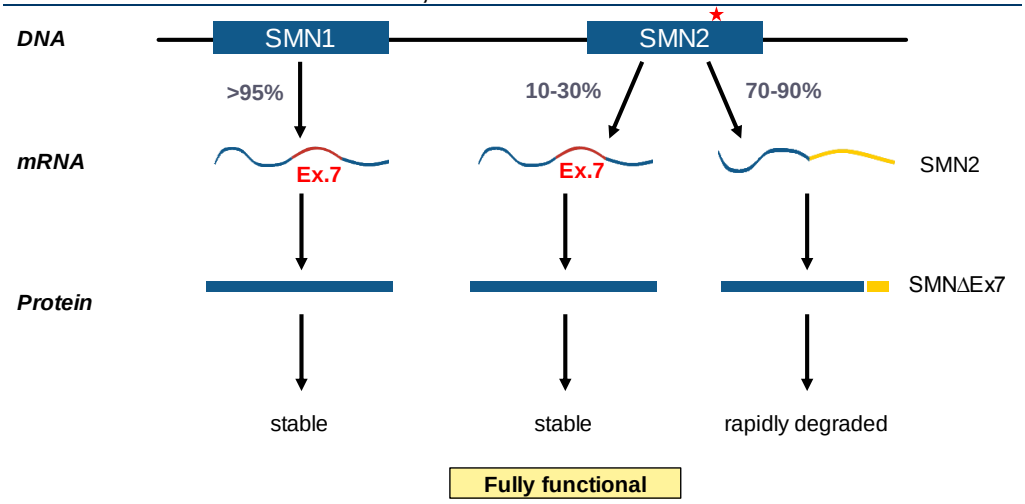
PTCT in collaboration with Roche and the SMA Foundation is developing drugs that increase the production of "normal" protein from the SMN2 gene. PTCT's lead candidates act on the SMN2 message and cause it preferentially to splice into the active form.

Normally, SMN2 is spliced correctly 10-30% of the time (see Exhibit 24), but in the presence of PTCT's compound, correct splicing occurs up to 95% of the time (see Exhibit 25), producing enough SMN protein significantly to alter the development of the disease in animal models of SMA.

Evidence for the efficacy of this compound comes from in vivo studies in disease models.

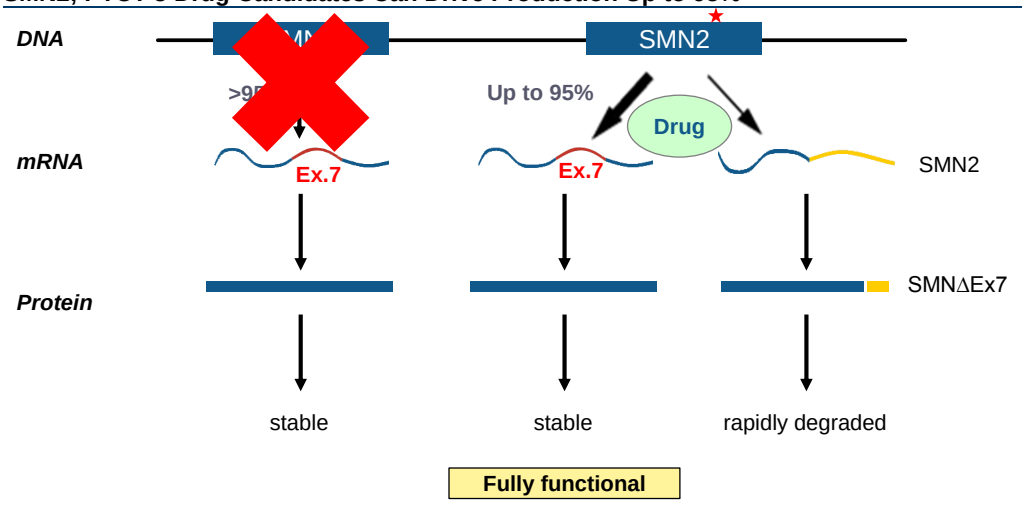
- **Evidence of Mechanism:** RNA from the brain or muscle of diseased mice shows that treatment with PTCT's SMA candidate increases production of the "normal" full-length RNA (see Exhibit 26, left panel) and increases production of the SMN protein in target tissues. (See Exhibit 26, right panel.)
- **Evidence of Activity:** In animal models of SMA, PTCT's candidates promote long-term survival, while untreated animals die within three weeks. (See Exhibit 27.)

Exhibit 24: Under Normal Conditions, SMN1 Produces Sufficient SMN Protein



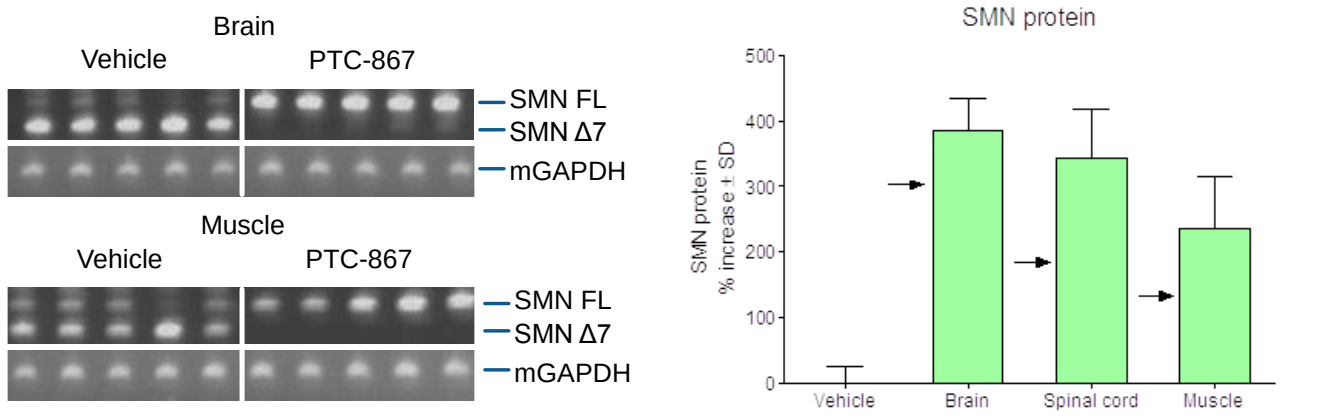
Source: Company data

Exhibit 25: When SMN1 Gene Function Is Lost, SMN Is Produced at Low Levels from SMN2; PTCT's Drug Candidates Can Drive Production Up to 95%



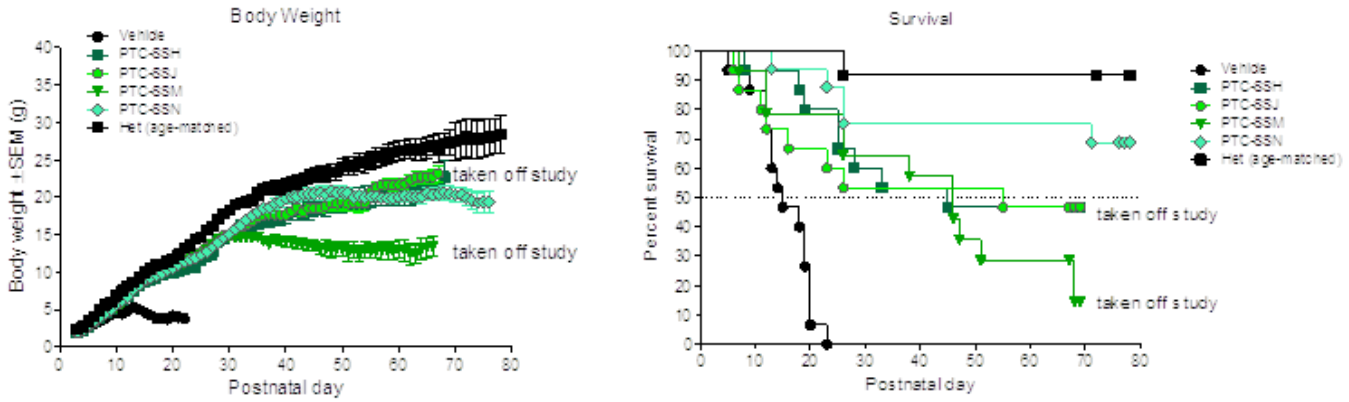
Source: Company data

Exhibit 26: Increased SMN RNA and Protein after Administration of PTCT's Drug Candidate



Source: Company data

Exhibit 27: Long-Term Dosing Leads to Weight Gain and Increased Survival with Different Drug Candidates



Source: Company data

Next Steps in the Program

We expect that PTCT and Roche will select a development candidate, most likely in Q3:13. This would trigger a $10M milestone payment to PTCT, which we already model in Q3.

Assuming preclinical toxicology is clean, we expect that the chosen candidate could enter human clinical testing sometime in 2014, likely triggering another cash milestone to PTCT.

Terms of the SMA Deal

PTCT and Roche entered into a deal in November 2011 for the worldwide rights to the SMA program. PTCT received $30M upfront and is eligible for $135M in development milestones and $325M in sales milestones. PTCT will receive royalties on WW net sales that are tiered in the single digits to the mid-teens.

Several Novel Drugs in Development for SMA

The molecular understanding of the cause of SMA has led several groups to develop novel approaches to increase SMN protein levels. (See Exhibit 28.) The most advanced

program is from Isis/Biogen's ISIS-SMNRx in Phase II. While PTCT is clearly behind, the field is still wide open, and the preclinical data so far from PTCT are compelling. Physicians we have spoken to who are familiar with these data are excited about the potential activity and its utility in SMA.

Exhibit 28: Drugs that Aim to Increase SMN Production

Company	Drug	MOA	Stage
ISIS/Biogen	ISIS-SMNRx	SMN2 splicing augmentation	Phase II
Pfizer	RG3039	Impact mRNA metabolism	Preclinical
PTC/Roche	Small molecule	SMN2 splicing augmentation	Preclinical
Genethon	AAV with SMN1	Gene therapy	Preclinical

Source: Company data, ClinicalTrials.gov

PTCT Management

Stuart Peltz, Ph.D. - Chief Executive Officer

Dr. Peltz co-founded PTCT in 1998 and has been the CEO since. Previously, Dr. Peltz was a Professor in the Department of Molecular Genetics & Microbiology at the Robert Wood Johnson Medical School at Rutgers University. Dr. Peltz received a Ph.D. from the McArdle Laboratory for Cancer Research at the University of Wisconsin.

Cláudia Hirawat - President

Ms. Hirawat has been PTCT's President since April 2012, and prior to that position she was Senior Vice President, Corporate Development at PTCT from 2006 to April 2012. She also served in other roles since joining the company in 2000. Previously, Ms. Hirawat was a Vice President at LedbetterStevens from 1995 to 2000.

Mark Rothera - Chief Commercial Officer

Mr. Rothera joined PTCT as CCO in April 2013. Previously, Mr. Rothera was the Global President of Aegerion Pharmaceuticals from 2012 to 2013. From 2006 to 2012, he served as Vice President and General Manager in Shire Human Genetic Therapies commercial operations division. Before Shire, Mr. Rothera held different commercial and operation roles at Glaxo Wellcome.

Mark Boulding - Executive Vice President and Chief Legal Officer

Mr. Boulding has been PTCT's Executive Vice President and Chief Legal Officer since March 2012, after serving as Senior Vice President and General Counsel from 2002 to 2012. Previously, Mr. Boulding served as General Counsel, Executive Vice President and Secretary of MedicaLogic/Medscape, Inc. 1999 to 2002. Mr. Boulding previously was a partner in two Washington, D.C.-based law firms.

Shane Kovacs - Chief Financial Officer

Mr. Kovacs joined PTCT in June 2013. Before PTCT, Mr. Kovacs served as Managing Director, Health Care Investment Banking at Credit Suisse. Before Credit Suisse, Mr. Kovacs worked in investment banking at National Bank Financial in Toronto.

Neil Almstead, Ph.D. - Senior Vice President, Research and CMC

Dr. Almstead has served as Senior Vice President, Research and CMC since July 2008 and was Senior Vice President, Chemistry and CMC from January 2007 to June 2008. Prior to joining PTCT, Dr. Almstead also was a Project Manager at Procter & Gamble Company.

Jay Barth, M.D. - Vice President, Clinical Development

Dr. Barth has been Vice President of Clinical Development at PTCT since January 2011, and before he served as Executive Director of Clinical Development from 2009 to 2010. Before PTCT, Dr. Barth was an Executive Director of Clinical Research at Merck from 2007 to 2008. From 2005 to 2007, he served as Vice President, Clinical Research and Medical Affairs at Altana Pharma US.

Financial Statements

Exhibit 29: PTCT Income Statement

($ in MM; except per share)	2011A	2012A	Q1:13A	Q2:13E	Q3:13E	Q4:13E	2013E	2014E	2015E	2016E	2017E	2018E
US Sales										15.6	102.2	174.7
EU Sales										13.7	51.8	114.4
ROW Royalties											2.7	7.0
Ataluren revenue (total)										29.3	156.6	296.1
Collaboration revenue	99.0	28.8	6.1	5.0	5.0	10.0	26.1	16.0	23.0	16.0	16.0	12.0
Grant revenue	6.5	5.2	1.1	1.0	1.0	1.0	4.1	4.0	4.0			
Total Revenues	105.4	33.9	7.1	6.0	6.0	11.0	30.1	20.0	27.0	45.3	172.6	308.1
COGS										2.3	12.5	23.7
Research and Development Expenses	58.7	46.1	11.3	11.0	11.5	12.0	45.8	50.7	53.8	63.0	70.0	77.0
Sales, General and Administrative Expenses	16.2	14.6	4.5	7.0	6.5	6.5	24.5	26.0	29.0	67.0	101.0	119.2
Total Costs and Expenses	74.8	60.8	15.7	18.0	18.0	18.5	70.2	76.7	82.8	132.3	183.5	219.9
Operating Income (Loss)	30.6	(26.8)	(8.6)	(12.0)	(12.0)	(7.5)	(40.1)	(56.7)	(55.8)	(87.1)	(10.9)	88.2
Interest Expense, net	(2.4)	(1.2)	(6.2)	(0.1)	(0.1)	(0.0)	(6.4)					
Other income, net	0.5	1.8	0.1	(1.0)	(1.0)	(1.0)	(2.9)	(4.0)	(4.0)	(4.0)	(4.0)	(4.0)
Income (Loss) before Tax	28.6	(26.2)	(14.7)	(13.1)	(13.1)	(8.5)	(49.4)	(60.7)	(59.8)	(91.1)	(14.9)	84.2
Provision for Income Tax (benefit)	2.3											
Net income (loss)	30.9	(26.2)	(14.7)	(13.1)	(13.1)	(8.5)	(49.4)	(60.7)	(59.8)	(91.1)	(14.9)	84.2
Net income attributable to common shareholders	0.0	0.7	(29.5)	(13.1)	(13.1)	(8.5)	(49.4)	(60.7)	(59.8)	(91.1)	(14.9)	84.2
EPS - diluted (proforma)	4.55	42.50	(2.08)	(0.86)	(0.52)	(0.34)	(2.26)	(2.19)	(1.64)	(2.44)	(0.39)	2.15
Shares Outstanding - basic (Proforma)	0.001	0.003	14.18	15.29	25.00	25.12	19.90	25.44	34.01	34.70	35.39	36.11
Shares Outstanding - diluted (Proforma)	0.006	0.017	16.10	17.22	27.02	27.24	21.89	27.67	36.42	37.31	38.22	39.17

Source: Company data, Credit Suisse estimates

Exhibit 30: PTCT Balance Sheet

Balance Sheet	2011A	2012A	Q1:13A	Q2:13E	Q3:13E	Q4:13E	2013E	2014E	2015E	2016E	2017E	2018E
Assets												
Cash, equivalents and marketable securities	**28.4**	**2.7**	**50.2**	172.1	155.7	149.4	149.4	90.7	228.9	149.8	146.9	243.1
Cash and cash equivalents	**28.4**	**2.7**	**50.2**	172.1	155.7	149.4	149.4	90.7	228.9	149.8	146.9	243.1
Marketable securities, available for sale, current portion	**0.0**	**0.0**	**0.0**	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Accounts receivable												
Prepaid expenses and other current assets	**3.4**	**0.9**	**0.9**	0.9	0.9	0.9	0.9	0.9	0.9	0.9	0.9	0.9
Grant and collaboration receivables, net	**1.2**	**1.0**	**0.9**	0.9	0.9	0.9	0.9	0.9	0.9	0.9	0.9	0.9
Total current assets	**33.1**	**4.6**	**52.1**	174.0	157.6	151.3	**151.3**	**92.5**	**230.7**	**151.7**	**148.7**	**244.9**
Fixed assets, net	**10.8**	**8.3**	**7.7**	7.1	6.4	5.8	5.8	3.3	2.5	1.7	0.9	0.1
Deposits and other assets	**0.3**	**0.2**	**0.2**	0.2	0.2	0.2	0.2	0.2	0.2	0.2	0.2	0.2
Other assets												
Total assets	**44.1**	**13.1**	**59.9**	**181.2**	**164.1**	**157.2**	**157.2**	**96.0**	**233.4**	**153.5**	**149.8**	**245.1**
Liabilities & Shareholders' Equity												
Accounts payable and accured expenses	**13.0**	**7.0**	**11.5**	10.5	9.5	8.5	8.5	5.5	5.5	5.5	5.5	5.5
Current portion of long-term debt	**7.1**	**4.4**	**3.8**	2.6	1.4	0.1	0.1	0.0	0.0	0.0	0.0	0.0
Deferred revenues, current portion	**23.0**	**16.7**	**12.0**	9.0	6.0	8.0	8.0	0.0	0.0	0.0	0.0	0.0
Tottal Current liabilities	**43.1**	**28.2**	**27.3**	**22.1**	**16.9**	**16.6**	**16.6**	**5.5**	**5.5**	**5.5**	**5.5**	**5.5**
Deferred revenues, excluding current portion	**16.4**	**0.7**	**0.5**	0.3	0.1	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Long-term debt	**4.5**	**0.4**	**0.0**	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Other liabilities, long-term	**4.2**	**2.5**	**2.5**	2.5	2.5	2.5	2.5	2.5	2.5	2.5	2.5	2.5
Total liabilities	**68.4**	**31.9**	**30.3**	**24.9**	**19.5**	**19.1**	**19.1**	**8.0**	**8.0**	**8.0**	**8.0**	**8.0**
Preferred stock	**214.4**	**80.8**	**158.1**	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Common stock, $0.01 par value; 216,666 shares authorized; 1	**0.0**	**0.0**	**0.0**	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Additional paid-in capital	**12.0**	**177.6**	**163.3**	461.3	462.8	464.8	464.8	475.4	672.6	683.8	695.0	706.2
Accumulated deficit	**(250.6)**	**(277.2)**	**(291.9)**	(305.0)	(318.1)	(326.7)	(326.7)	(387.4)	(447.2)	(538.2)	(553.2)	(469.0)
Total stockholders' equity	**(24.2)**	**(18.8)**	**29.6**	**156.3**	**144.7**	**138.1**	**138.1**	**88.0**	**225.4**	**145.5**	**141.8**	**237.2**
Total liabilities and stockholders' equity	**44.1**	**13.1**	**59.9**	**181.2**	**164.2**	**157.2**	**157.2**	**96.0**	**233.4**	**153.5**	**149.8**	**245.1**

Source: Company data, Credit Suisse estimates

Exhibit 31: PTCT Cash Flow Statement

Cash Flow Statement	2011A	2012A	Q1:13A	Q2:13E	Q3:13E	Q4:13E	2013E	2014E	2015E	2016E	2017E	2018E
Cash flows from operating activities:												
Net income (loss)	**30.9**	**(26.2)**	**(14.7)**	(13.1)	(13.1)	(8.5)	**(49.4)**	**(60.7)**	**(59.8)**	**(91.1)**	**(14.9)**	**84.2**
Adjustments to reconcile net loss to net cash used in operating activities:												
Depreciation	**2.9**	**2.7**	**0.6**	0.6	0.6	0.6	**2.5**	**2.5**	**0.8**	**0.8**	**0.8**	**0.8**
Change in valuation of warrant liability	**(0.5)**	**(1.8)**	**(0.1)**	0.0	0.0	0.0	**(0.1)**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**
Noncash interest expense	**0.4**	**0.2**	**6.0**	0.0	0.0	0.0	**6.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**
Stock-based compensation expense	**2.8**	**2.3**	**0.6**	1.0	1.5	2.0	**5.1**	**10.6**	**11.2**	**11.2**	**11.2**	**11.2**
Changes in operating assets and liabilities:												
Accounts receivable												
Prepaid expenses and other current assets	**(1.4)**	**2.5**	**(0.0)**	0.0	0.0	0.0	**(0.0)**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**
Grant and collaboration receivables	**2.1**	**0.2**	**0.1**	0.0	0.0	0.0	**0.1**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**
Deposits and other assets	**0.2**	**0.1**	**0.0**	0.0	0.0	0.0	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**
Accounts payable and accrued expenses	**(3.2)**	**(6.0)**	**4.4**	(1.0)	(1.0)	(1.0)	**1.4**	**(3.0)**	**0.0**	**0.0**	**0.0**	**0.0**
Other long-term liabilities	**(0.1)**	**0.1**	**0.0**	0.0	0.0	0.0	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**
Deferred revenue	**(55.0)**	**(22.0)**	**(4.9)**	(3.2)	(3.2)	1.9	**(9.4)**	**(8.0)**	**0.0**	**0.0**	**0.0**	**0.0**
Net cash used in operating activities	**(20.8)**	**(47.9)**	**(7.8)**	**(15.7)**	**(15.2)**	**(5.0)**	**(43.7)**	**(58.6)**	**(47.8)**	**(79.1)**	**(2.9)**	**96.2**
Cash flows from investing activities												
Purchases of fixed assets	**(0.2)**	**(0.2)**	**(0.0)**	0.0	0.0	0.0	**(0.0)**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**
Purchases of investments	**(2.0)**	**0.0**	**0.0**				**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**
Maturities of investments	**29.9**	**0.0**	**0.0**				**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**
Net cash used in investing activities	**27.7**	**(0.2)**	**(0.0)**	**0.0**	**0.0**	**0.0**	**(0.0)**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**
Cash flows from financing activities:												
Payments on long-term debt	**(7.2)**	**(6.9)**	**(1.1)**	(1.2)	(1.2)	(1.3)	**(4.8)**	**(0.1)**	**0.0**	**0.0**	**0.0**	**0.0**
Net proceeds from sale of preferred stock	**0.0**	**29.4**	**56.5**	4.5			**61.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**
Proceeds from issuance of common stock	**0.0**	**0.0**	**0.0**	134.3	0.0	0.0	**134.3**	**0.0**	**186.0**	**0.0**	**0.0**	**0.0**
Net cash (used in) provided by financing activities	**(7.2)**	**22.4**	**55.4**	**137.6**	**(1.2)**	**(1.3)**	**190.5**	**(0.1)**	**186.0**	**0.0**	**0.0**	**0.0**
Net increase (decrease) in cash and cash equivalents	**(0.2)**	**(25.7)**	**47.5**	**121.9**	**(16.4)**	**(6.3)**	**146.7**	**(58.7)**	**138.2**	**(79.1)**	**(2.9)**	**96.2**
Cash and cash equivalents—beginning of period	**28.7**	**28.4**	**2.7**	50.2	172.1	155.7	**2.7**	**149.4**	**90.7**	**228.9**	**149.8**	**146.9**
Cash and cash equivalents—end of period	**28.4**	**2.7**	**50.2**	**172.1**	**155.7**	**149.4**	**149.4**	**90.7**	**228.9**	**149.8**	**146.9**	**243.1**

Source: Company data, Credit Suisse estimates

Companies Mentioned *(Price as of 12-Jul-2013)*

Aegerion (AEGR.OQ, $77.41)
Alexion Pharmaceuticals Inc. (ALXN.OQ, $114.26)
Alnylam Pharm (ALNY.OQ, $50.04)
Amicus (FOLD.OQ, $2.36)
BioMarin Pharmaceutical, Inc. (BMRN.OQ, $64.81)
Biogen Idec (BIIB.OQ, $226.34)
Corcept Therapeutics (CORT.OQ, $1.8)
GlaxoSmithKline plc (GSK.L, 1749.0p)
Isis Pharma (ISIS.OQ, $33.72)
PTC Therapeutics, Inc (PTCT.OQ, $16.94, OUTPERFORM[V], TP $24.0)
Pfizer (PFE.N, $28.81)
Prosensa (RNA.OQ, $27.75)
Raptor Pharmactl (RPTP.OQ, $10.77)
Roche (ROG.VX, SFr243.8)
Sarepta Theprcs (SRPT.OQ, $44.33)
Synageva Bio (GEVA.OQ, $47.15)
Vertex Pharmaceuticals Inc. (VRTX.OQ, $87.79)

Disclosure Appendix

Important Global Disclosures

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Price Target: (12 months) for PTC Therapeutics, Inc (PTCT.OQ)

Method: Our $24 target price for PTCT is calculated by DCF (discounted cash flow), using probability-weighted sales estimates for ataluren in Duchenne muscular dystrophy (60% probability) and in cystic fibrosis (60% probability) modeled through 2030. We use a 38% tax rate and a 12% discount rate, and arrive at a $30 valuation based on current share count. We conservatively assume that PTCT will raise additional capital in 2015 and therefore adjust our valuation by adding 5 to 8M additional shares, which gives us a $24 target price.

Risk: Risks to our $24 target price for PTCT are (1) unexpected negative result in the Duchenne muscular dystrophy (DMD) or cystic fibrosis (CF) Phase III studies, (2) headline risk should the EMA (European Medicines Agency) reject conditional approval of ataluren in DMD, (3) limited newsflow in 2014, (4) potential emergence a competitive molecule in the DMD or CF space, and (5) potential need for additional capital (we model an equity raise in 2015).

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