



Vertex Pharma (NAS: VRTX)

Industry: Biotechnology
NAICS Code: 325414

Current Price: \$350
DCF Valuation: \$341
Potential upside: -3%
Investment Recommendation: **Hold**

Major assumption drivers

- Our sales forecast is dependent on successful product launches of new products Exa-cel® and Venzacافتور® in the next two years
- Sales are also dependent on the establishment of a mutually beneficial reimbursement system for payment of gene-based therapies by different healthcare payers within the next 3 years

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Report Date: December 6th, 2023

Vertex Pharmaceuticals Overview

Vertex Pharmaceuticals: Vertex Pharmaceuticals is a global biotechnology company focusing on the discovery of small molecule drugs for cystic fibrosis, transfusion-dependent beta-thalassemia, and sickle cell disease. Vertex remains the market leader in cystic fibrosis therapies, having developed five key drugs for it. Vertex's focus on new indications is an attempt to diversify its pipeline outside of cystic fibrosis.

Summary of Vertex Pharma Key Products

Kalydeco, Orkambi, Symkevi/symdeko, Kaftrio/Trikafta and Vanzacaftor are Vertex's 5 main drugs focused on the treatment of cystic fibrosis. Exa-cel is a drug focusing on transfusion-dependent beta-thalassemia and sickle cell disease.

Cystic Fibrosis Pipeline



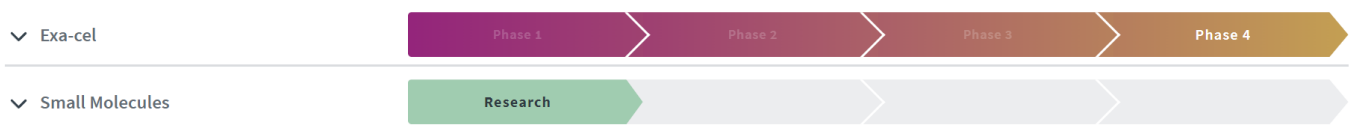
Hematology Pipeline



Sickle cell disease

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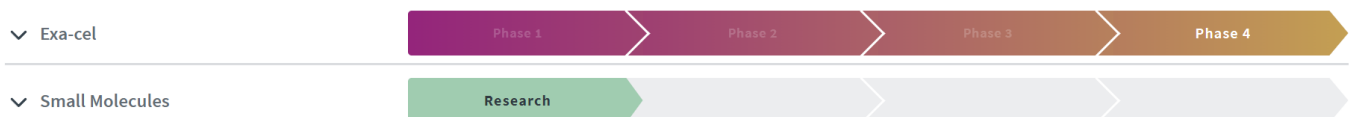
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Beta thalassemia

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Summary from Q3 2023 earnings call on November 6, 2023

In Q3 2023, Vertex Pharmaceuticals boasted a product revenue of \$2.48B corresponding to a 6% increase relative to the previous quarter. The company's full-year product revenue target increased to ~\$9.85B from \$9.75B and the company's pipeline continued to develop with Phase 3 data in cystic fibrosis and acute pain expected in early 2024 as well as Phase 2 data in neuropathic pain expected in late 2023.

Competitive Landscape

Cystic Fibrosis – Currently no other Biotech company produces any medications that can be used as a disease modifying agent for cystic fibrosis. Boehringer Ingelheim and Abbvie have products in their pipeline in phase 1 and 2 respectively. These two products will have no impact on projections for the next 5 years.

Hematology- Exa-cel® is going to be the first gene based therapy for the cure of sickle cell disease, all other medications are used for long term management, specifically for management and reduction of Vaso-occlusive crises. These medications although used for the same disease are intended for different treatment outcomes and so are not direct competitors.

Income Statement Metrics

Sales forecast

Vertex Pharmaceuticals currently only sells medicines that serve cystic fibrosis patients, the company currently has 4 FDA-approved products in the market and plans to add a new product (Vanzacaftor®) in Q1 2024. In addition, on the 8th of December 2023, we expect a decision from the FDA that will open up a new business line for the company. Exa-cel® is expected to receive the market authorization from the FDA in Dec 2023. To forecast sales, we considered the sales trends we expect over the next 5 years for the 5 different products in the Cystic fibrosis franchise and the sales we expect for the new Hematologic franchise set for launch in 2024.

Cystic Fibrosis Franchise Sales

Vertex is the only company that makes drugs that effectively manage Cystic fibrosis, and so they enjoy a monopoly over the market. We forecasted the overall market opportunity based on epidemiologic trends in the disease. Table 1 shows the trends we expect in the disease's Global prevalence; the number of people living with the disease worldwide and US+EU prevalence data; the number of people living with the disease in the US and EU.

We forecast the prevalence of the disease to increase by about ~2000 patients per year after accounting for deaths from the disease. Our assumptions are based on the EU incidence rate of 0.737/ 10,000 people annually¹ and the ~1000 new patients diagnosed in the US annually².

Table 1

Epidemiologic Data Estimates	2018A	2019A	2020A	2021A	2022A	FY2023	2024E	2025E	2026E	2027E	2028E
Global Prevalence	150,388	152,728	155,068	157,408	159,748	162,088	163,748	165,408	167,068	168,728	170,388
US + EU Prevalence	64,810	67,150	69,490	71,830	74,170	76,510	78,170	79,830	81,490	83,150	84,810

To determine how many of these patients Vertex was going to serve over the next 5 years we looked at recent growth in the number of patients that are currently on Vertex medications. Growth has slowed as the market has become more mature but patient attrition rates remain low³. We project a modest 5% growth rate in the number of new patients that will be placed on the drug based on the most recent sales growth trend.

Vertex Pharma has a product strategy of cannibalizing older products every few years for new products, since the launch of Trikafta® we have seen a 25%, 17%, and 11% YoY decline in revenues for Symdeko®, Orkambi®, and Kalydeco® respectively. We project that these declines will continue over the next 5 years.

A new product is set to launch in Q1 2024 called Vanzacaftor®, management claims this product will be better than Trikafta® in terms of clinical effectiveness and offers improved patient convenience through once-daily dosing vs twice daily. To project the sales for this new product and the market share capture from Trikafta® we looked at the market share capture Trikafta® had in 2019 vs the 3 older brands and

¹ Farrell, "The Prevalence of Cystic Fibrosis in the European Union."

² "About Cystic Fibrosis | Cystic Fibrosis Foundation."

³ Lisa et al., "CORPORATE PARTICIPANTS."

projected a similar trend for patients switching to the more effective therapy in 2024. The estimated price of Vanzacaftor® is expected to be 15% higher than Trikafta at \$369K vs \$322K based on the most recent price increase at the launch of Trikafta in 2019. Based on all these assumptions sales for the entire franchise are summarized in table 3 below.

Table 2

Cystic fibrosis Patient data	2018A	2019A	2020A	2021A	2022A	FY2023	2024E	2025E	2026E	2027E	2028E
# of Patients on Older Brands(Symdeko, Orkambi and Kalydeco)	10,818	13,213	8,290	6,671	4,419	3,731	3,160	2,683	2,285	1,950	1,668
SYMDEKO/ SYMKEVI	2,589	4,771	2,114	1,414	606	453	339	254	190	142	106
ORKAMBI	4,873	5,139	3,502	2,977	1,969	1,643	1,370	1,143	954	796	664
KALYDECO	3,357	3,303	2,673	2,280	1,843	1,635	1,450	1,287	1,141	1,012	898
# of patients Newer Brands (Trikafta + Vanzacaftor)	-	1,304	11,997	17,693	23,870	27,560	29,751	31,930	34,120	36,338	38,601
TRIKAFTA/ KAFTRIO	-	1,304	11,997	17,693	23,870	27,560	27,078	13,048	9,342	5,676	4,603
Vanzacaftor (steal from Trikaftor)							2,673	18,882	24,777	30,662	33,999
Total Number of patients on Vertex meds	10,818	14,518	20,287	24,364	28,288	31,291	32,910	34,613	36,404	38,288	40,269
Market share capture (Trikaftor vs Older brands)		9%	59%	73%	84%	88%					

Table 3

Cystic Fibrosis Franchise	2018A	2019A	2020A	2021A	2022A	FY2023	2024E	2025E	2026E	2027E	2028E
TRIKAFTA/ KAFTRIO	0	420	3863	5697	7686	8,874	8,719	4,201	3,008	1,828	1,482
SYMDEKO/ SYMKEVI	769	1417	628	420	180	135	101	75	56	42	32
ORKAMBI	1262	1331	907	771	510	425	355	296	247	206	172
KALYDECO	1007	991	802	684	553	491	435	386	342	304	269
Vanzacaftor							987	6,972	9,149	11,322	12,554
Total CF Sales	3,038	4,159	6,200	7,572	8,929	9,925	10,597	11,931	12,803	13,701	14,509

Hematology Franchise

We based our sales projections for Exa-cel® on epidemiologic trends to determine market size. We estimated the number of patients with the disease based on the prevalence and incidence of sickle cell disease in the US and the EU. Current estimates of the prevalence of sickle cell disease in the US are ~100,000 patients⁴, and ~52,000 patients in the EU⁵. With incidence rates that amount to ~1400 and ~1700 new patients/ year respectively.

Table 4

Epidemiologic Data	FY2023	2024E	2025E	2026E	2027E	2028E
Incidence rate of Sickle cell disease in the US	1,416					
US prevalence of Sickle cell disease	100,000	101,264	102,010	102,045	102,183	102,319
Incidence rate of Sickle cell disease in the EU	1,696					
Prevalence rate of SCD in the EU	52000	53,258	54,322	55,002	55,729	56,447

The product will be the first CRISPR-based gene editing medication to be launched since the technology won a Nobel Prize in 2020. The product is expected to debut at a price between \$2M and \$4M we forecasted with a price tag of \$2.8M based on a similar product launched by Novartis for the treatment of Spine Muscular Atrophy . This price tag limited the number of benchmarks available for modeling patient uptake, however, we modeled patient uptake based on another rare gene therapy launched in 2019 by Novartis for the treatment of Spinal Muscular Atrophy (SMA), this product debuted at \$2.8M and has a similar patient population. In the first 5 years of launch, patient uptake for Zolgensma® is shown in Table 5 below. We modeled a similar patient uptake for Exa-cel®. The last assumption that is built into our projected sales for Exa-cel® is the impact of the planned new Medicaid pricing model for gene-based therapies which is scheduled to go live in 2026, this pricing model will make the product accessible to a lot of African Americans who are more genetically predisposed to the disease, we expect uptake to increase by over 60% based on trend observed in Sovaldi/Harvoni uptake after a pricing mechanism was established for the Hepatitis C medications.

⁴ CDC, "Data & Statistics on Sickle Cell Disease | CDC."

⁵ Colombatti and Sainati, "Management of Children With Sickle Cell Disease in Europe."

Table 5

	2019A	2020A	2021A	2022A	FY2023
revenue	210	459	469	434	
No of patients	100	219	223	207	
Patient uptake for new gene therapies (Based on Zolgensma)	0.30%	0.66%	1.35%	1.25%	1.25%

Table 6

Exa-cel	2024E	2025E	2026E	2027E	2028E
Sickle cell Disease	424	1,878	3,866	3,579	3,584
Beta Thalassemia	59	130	265	245	245
Sickle Cell Disease (EU only)	-	988	2,059	1,929	1,954
Total Exa-cel Sales	484	2,996	6,190	5,753	5,784

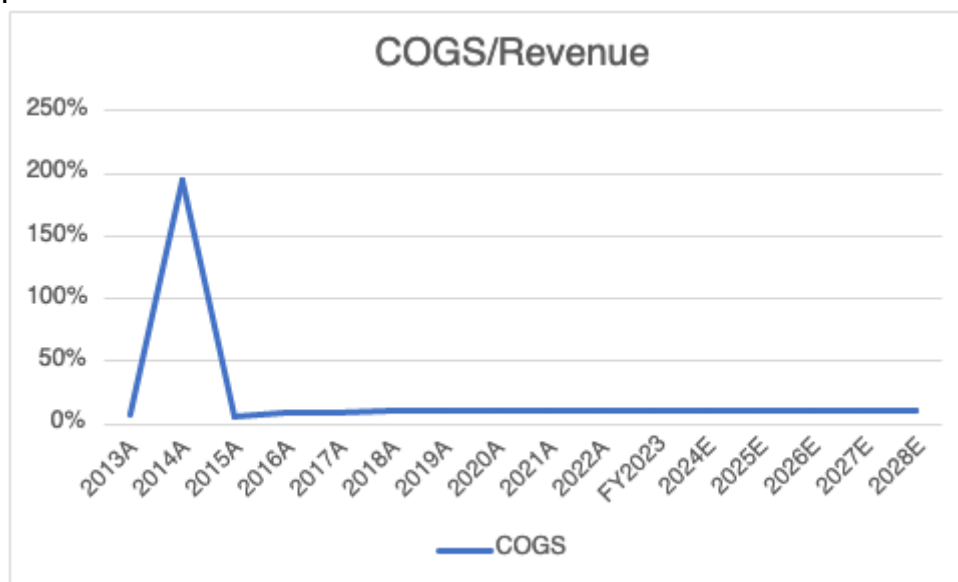
Total sales from both Franchises is shown below;

Table 7

	2018A	2019A	2020A	2021A	2022A	FY2023	2024E	2025E	2026E	2027E	2028E
Cystic Fibrosis Franchise Sales	3,038	4,159	6,200	7,572	8,929	9,925	10,597	11,931	12,803	13,701	14,509
Exa-cel Sales	-	-	-	-	-	-	484	2,996	6,190	5,753	5,784
Total Sales	3,038	4,159	6,200	7,572	8,929	9,925	11,080	14,926	18,993	19,455	20,292

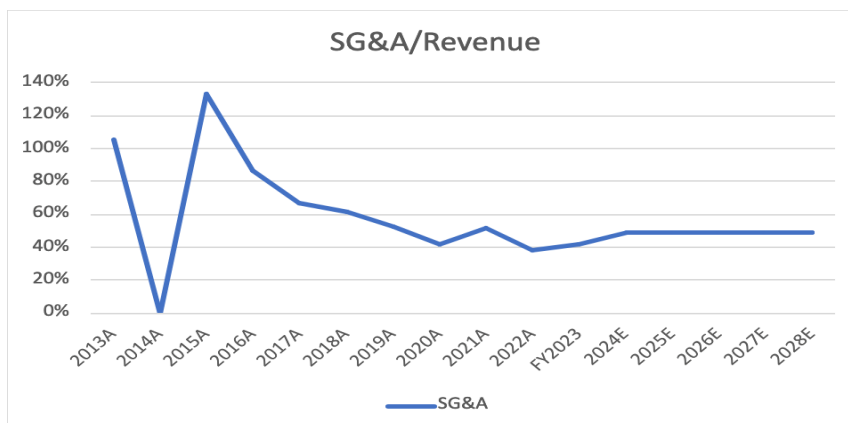
COGS

We forecasted COGS as a % of revenue, we project that COGS will remain relatively constant at 10% over the next 5 years.



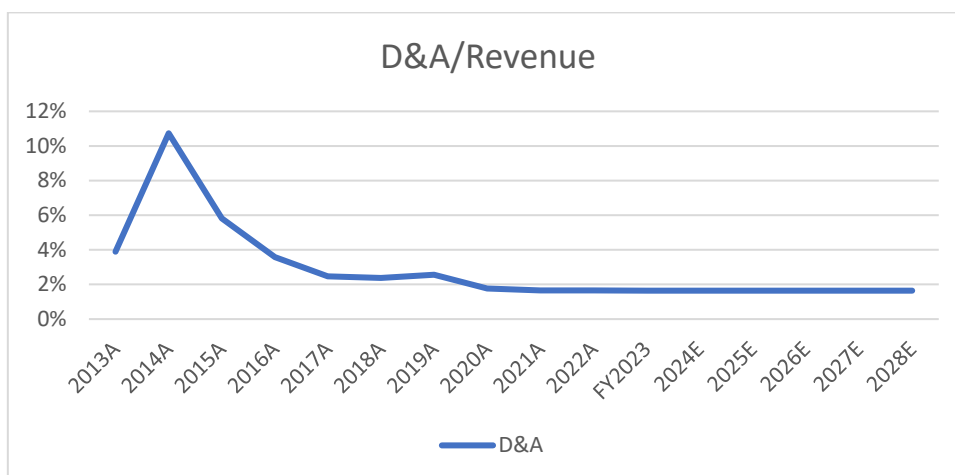
SG&A

We forecast SG&A as a proportion of revenue, SG&A has reduced significantly as the business has matured, we expect that SG&A will remain constant at ~47%, this is in line with other competitors in the industry.



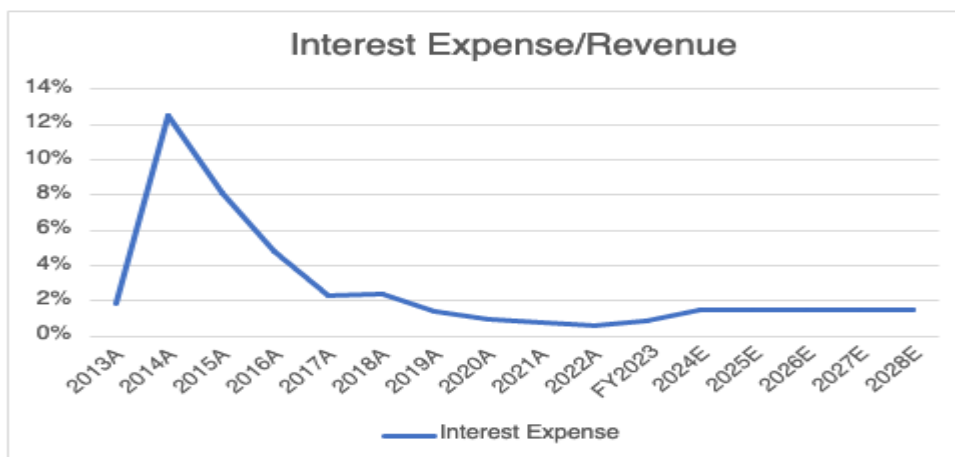
D&A

We forecast the D&A as a % of revenue, and forecast that it remains relatively constant over the next 5 years at 2%.



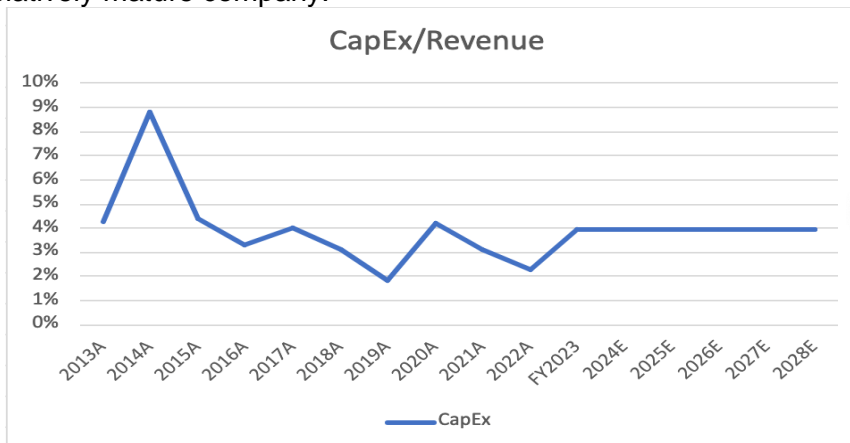
Interest Expense

The company currently has no publicly issued debt, the only debt it has is lease agreements till 2038 which constitute fixed annual payments. We forecast the interest payments to remain relatively constant over the next 5 years at ~1%.



CapEx

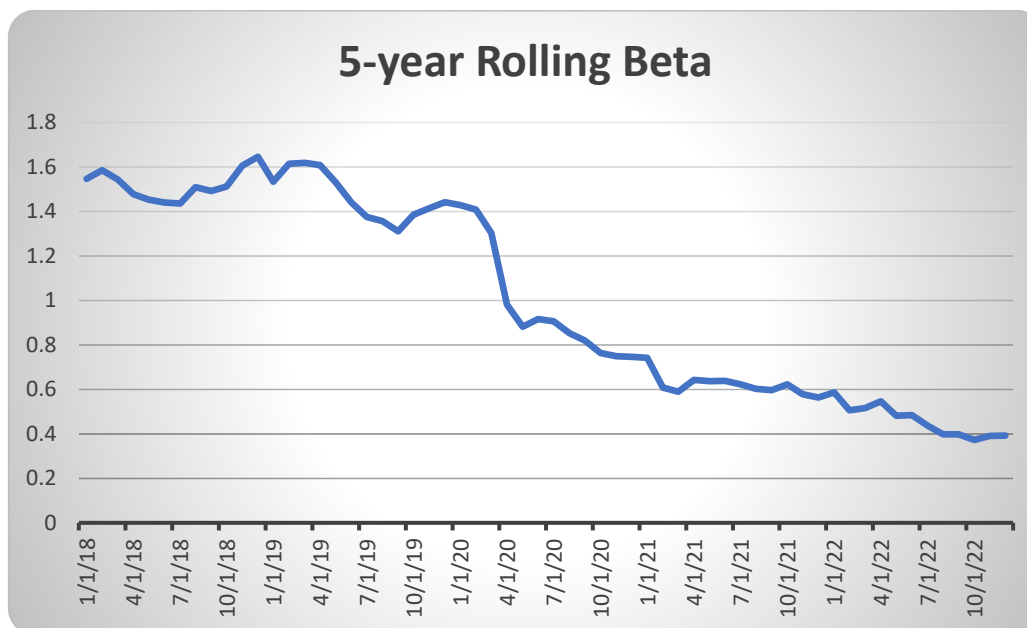
We forecast Capex as a proportion of revenue, and expect this value to remain constant at 4% per year given that this is a relatively mature company.



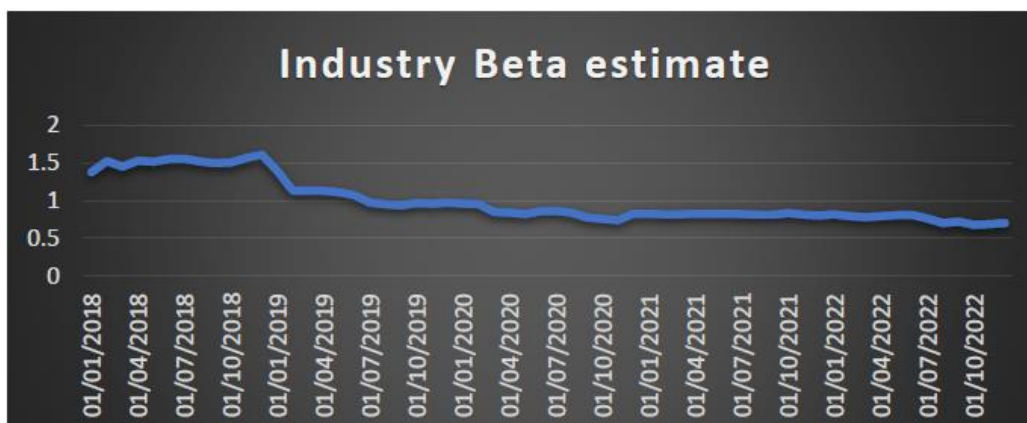
Beta Estimation

We used the industry beta of 0.688 for our valuation, this is because the company beta has been declining over the past 5 years and so we judged the beta estimate is unstable.

Company Beta



Industry Beta



Free Cash Flows Valuation

The company has no debt and so even though the Debt to Equity ratio has not remained constant over time we can value the unlevered free cash flows using the WACC method because there is no Debt-tax shield. Essentially, $WACC = r_A$.

$$r_E = r_A \left[1 + \frac{D}{E} \left(1 - \frac{T_C r_D}{1 + r_D} \right) \right]$$

Valuation Inputs

Tax Rate	21%
Risk-free rate	4.6%
Market Risk Premium	6.0%
Company Beta	0.69
Cost of Capital	7.1%
return on Assets	7.1%
Terminal Growth rate	3.00%

$$FCF = (EBIT + Non - op. Income) * (1 - T_c) + D\&A - CAPEX - \Delta WC$$

	FY2023	2024E	2025E	2026E	2027E	2028E	Terminal
<i>EBIT (\$M)</i>	4,603	5,295	7,132	9,076	9,296	9,697	
<i>EBIT x (1- Tax Rate) (\$M)</i>	3,659	4,209	5,670	7,215	7,391	7,709	
<i>D&A (\$M)</i>	163	222	299	380	389	406	
<i>CAPEX (\$M)</i>	395	441	593	755	773	807	
<i>Change in WC (\$M)</i>	1,705	1,904	2,564	3,263	3,342	3,486	
<i>FCF (\$M)</i>	1,722	2,087	2,811	3,577	3,664	3,822	
<i>Disount Factor</i>	1.00	1.07	1.15	1.23	1.31	1.41	
<i>Present Value</i>	1,722	1,949	2,452	2,914	2,787	2,715	73,520.08

Valuation Summary

Enterprise Value (\$M)	88,060.15
Share Price (\$)	341.32
Current Market Cap (\$M)	90,351.00
No of Outstanding Shares	258.00
Current share price	350
Upside potential	-3%