

# Vertex Pharmaceuticals

Nasdaq: VRTX



# Agenda

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# Investment Thesis

**1**

## **Further room for expansion within their CF treatments**

- Currently have one phase III trial ongoing
- Trikafta pending approval for children aged 2-5 years old, date set for April 28, 2023
- Treat remaining 5,000 patients (ineligible for CFTRm) with mRNA therapy
- 70,000 (2012) vs 105,000 (2022) people worldwide with CF
- Estimated 1000-2500 cases each year

**2**

## **Collaboration with CRISPR Therapeutics**

- Announced submission of their gene-therapy product (exa-cel) for beta-thalassaemia and sickle cell anaemia in the USA, EU and UK with a decision expected in 8 to 12 months

# CRISPR Technology

- Sickle-cell anaemia is a genetic disease affecting 100,000 people in the U.S.
  - Lifetime costs for sickle-cell is between \$4-6MM
- **Vertex estimates that approval of this treatment would be a multi-billion dollar opportunity**
  - Each treatment would likely cost several million dollars - still cost efficient for these patients
- This would make them the first company to bring therapy utilizing CRISPR technology to market which would come with a lot of publicity

# Company Overview

- Vertex Pharmaceuticals, established in 1989, is a leading global biotechnology company headquartered in Boston, Massachusetts. Vertex is focused on discovering, developing, and commercializing innovative therapies for serious diseases with a primary emphasis on cystic fibrosis (CF), a rare genetic disease that affects the lungs and other organs
- Their leading product is Trikafta(\$7.6B)
  - Orkambi(\$510M)
  - Kalydeco(\$553M)
  - Symdeco(\$180M)
- Trikafta is the first approved treatment that is effective for CF patients 12 years and older with at least one F508del mutation, which affects 90% of the population with CF



# Management Team



**CEO & President**

**Reshma Kewalramani, M.D., FASN**

- Joined Vertex in 2017
- 15+ years developing medicines
- She leads and executes the company's strategy



**CSO, VP**

**David Altshuler, M.D., Ph.D.**

- Joined Vertex in 2015
- Board of directors from 2012 to 2014
- Responsible for shaping the research pipeline



**COO, VP**

**Stuart A. Arbuckle**

- Joined Vertex in 2012
- Oversees Vertex's global commercial team

# Financials

	Revenues by Product				
	2022	% Change	2021	% Change	2020
	(Values in millions)				
Trikafta/Kaftrio	\$ 7,686.8	35%	\$ 5,697.2	47%	\$ 3,863.8
Symdeko/Symkevi	\$ 180.0	-57%	\$ 420.4	-33%	\$ 628.6
Orkambi	\$ 510.7	-34%	\$ 771.6	-15%	\$ 907.5
Kalydeco	\$ 553.2	-19%	\$ 684.2	-15%	\$ 802.9
Product Revenues	\$ 8,930.7	18%	\$ 7,573.4	22%	\$ 6,202.8

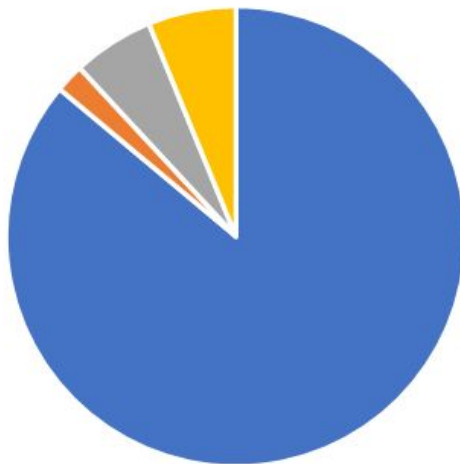
kalydeco

symdeko™

ORKAMBI®

trikafta™

Revenues by Product



■ Trikafta/Kaftrio ■ Symdeko/Symkevi ■ Orkambi ■ Kalydeco

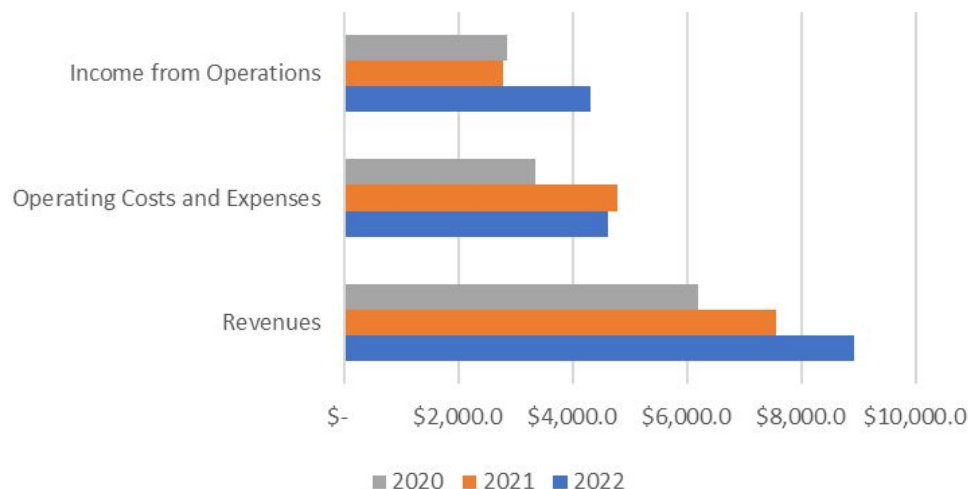
## Patent Expirations:

- **Trikafta:** There are **25 US patents**. They begin to expire in 2025, with generic entry being allowed in **December of 2037**
- **Symdeko:** **20 US patents**; they begin expiring in 2025 with generic entry being allowed in **April of 2035**
- **Orkambi:** **20 US patents**; they begin expiring in 2026, with generic entry allowed in **December of 2030**
- **Kalydeco:** **11 US patents**; patents begin expiring in 2025, with generic entry set to begin in **August of 2029**

# Financials (Operations)

	Results of Operations				
	2022	% Change	2021	% Change	2020
	(Values in Millions, except Percentages and Per Share Amounts)				
Revenues	\$ 8,930.7	18%	\$ 7,574.4	22%	\$ 6,205.7
Operating Costs and Expenses	\$ 4,623.3	-4%	\$ 4,791.3	43%	\$ 3,349.4
Income from Operations	\$ 4,307.4	55%	\$ 2,783.1	-3%	\$ 2,856.3
Other Non-Operating (Expense) Income, net	\$ (75.0)		\$ (51.7)		\$ 260.6
Provision for Income Taxes	\$ 910.4	134%	\$ 388.3	-4%	\$ 405.2
Net Income	\$ 3,322.0	42%	\$ 2,343.1	-14%	\$ 2,711.7
Net Income per Diluted Common Share	\$ 12.82		\$ 9.02		\$ 10.29
Diluted Shares Used	\$ 259.1		\$ 259.9		\$ 263.4

Operations over last 3 years (millions)

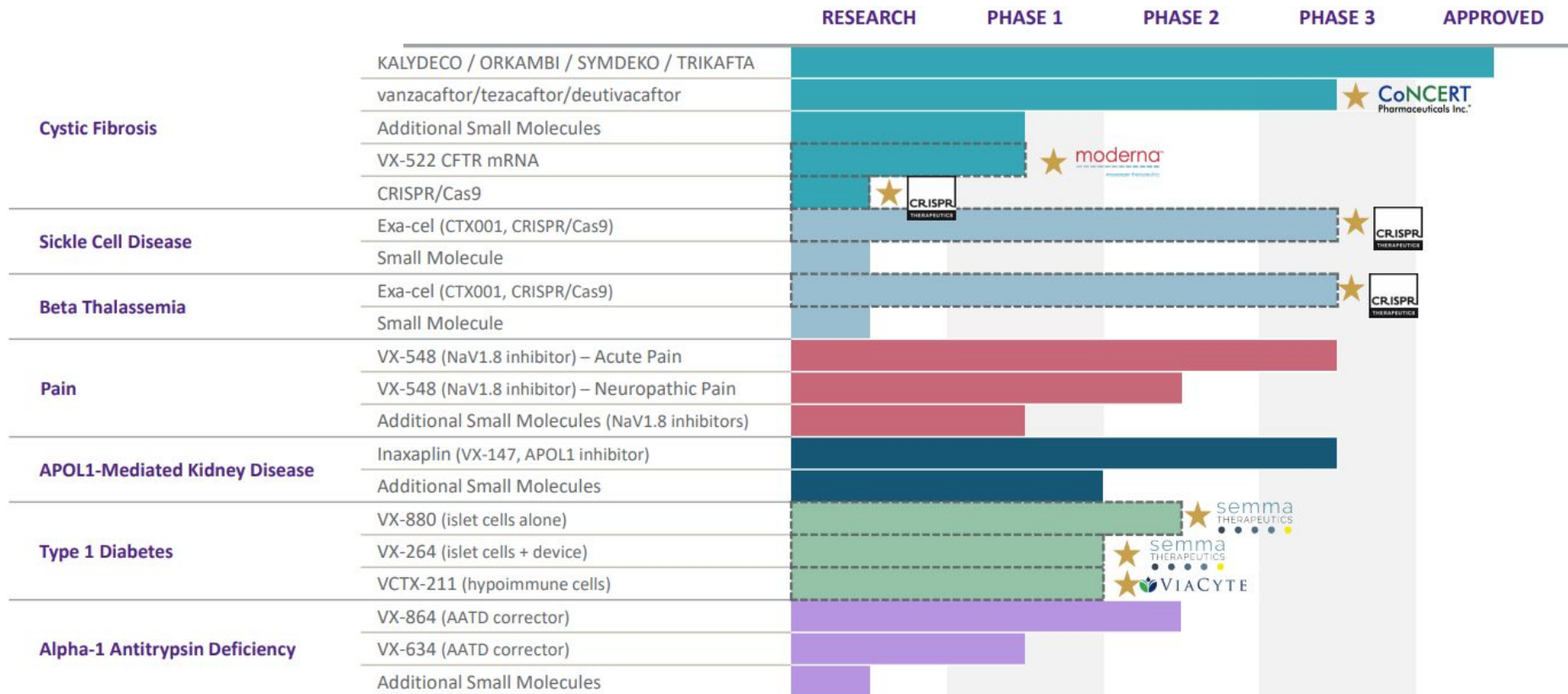


## Takeaways:

- Consistent **revenue growth** paired with a decrease in costs during 2022 has led to an increase in Net Income
- R&D Expenses have increased due to **expansion in cell and genetic therapy capabilities**
- That will continue as Vertex focuses on **transformative medicines** for serious diseases

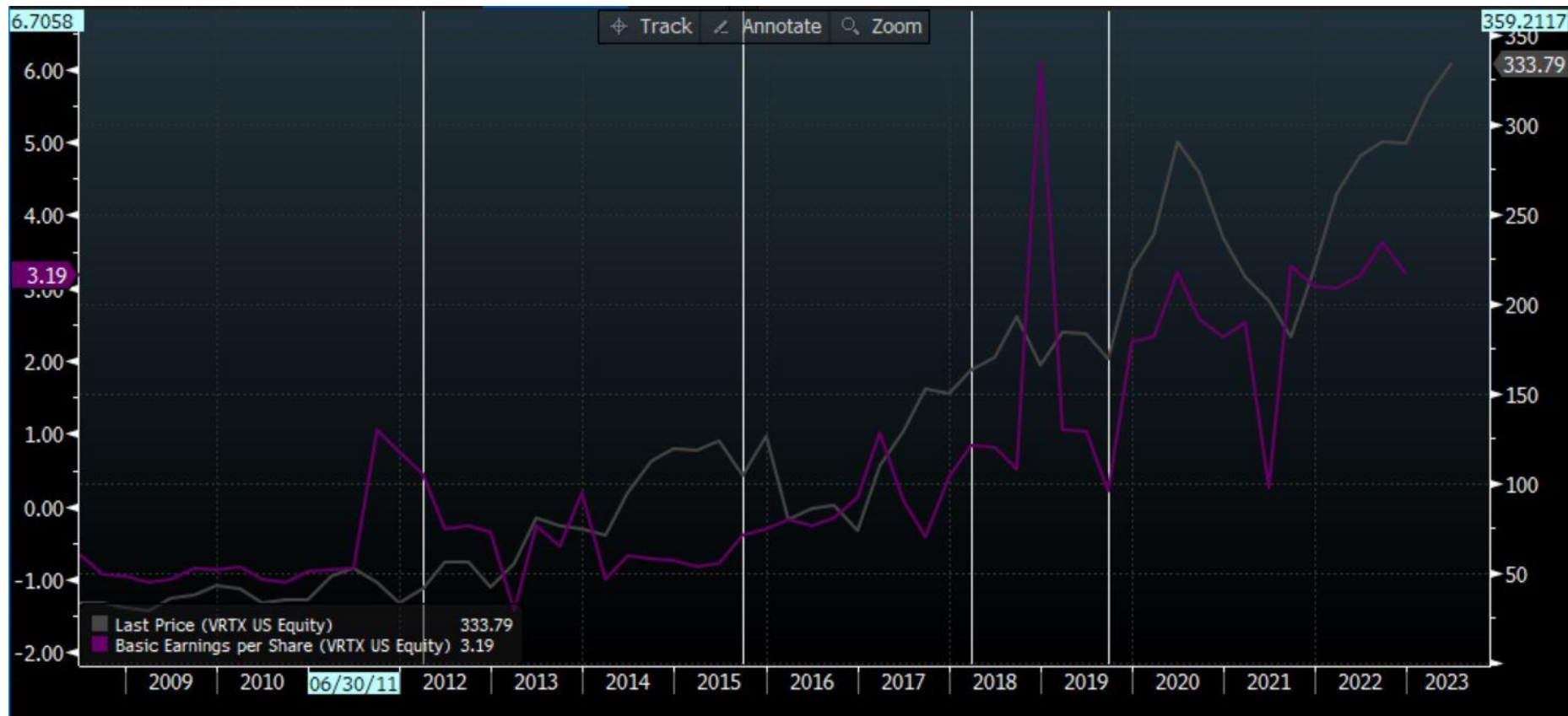


# Pipeline



Cell therapy or nucleic acid therapy (mRNA, gene editing) ★ Complementary BD

# Price Reaction to FDA Approval



# Industry Overview

- Vertex is heavily involved in the biotech and pharmaceutical industries
- **Bio-Tech**
  - \$860 billion industry
  - Projected \$1,683 billion by 2030
- **Pharmaceuticals**
  - \$1.42 trillion industry
- FDA has been placing holds on human trials to ensure proper protocols are followed in new studies
  - **Growth could slow across the industry** as testing will take longer
- CRISPR and gene editing have emerged at the cutting edge of biotechnology with the potential to eradicate genetic diseases

# Industry Overview



## AbbVie Inc

- What They Do: AbbVie produces drugs, most notably Humera which is used to treat Crohn's disease and rheumatoid arthritis
- Market Cap: \$285.24 B



## Krystal Biotech Inc

- What They Do: Krystal Biotech develops corrective and redosable gene therapy
- Market Cap: \$2.30 B



## Arcturus Therapeutics Holdings Inc

- What They Do: Arcturus develops therapeutics for rare and infectious diseases
- Market Cap: \$742.76 MM



## 4D Molecular Therapeutics Inc

- What They Do: 4D designs genetic medicines for rare and large market diseases
- Market Cap: \$643.75 MM

# Industry Overview

## Biotechnology

- Artificial Intelligence
  - AI can execute massive tasks leading to more breakthroughs
- Big Data
  - The rise of big data has enabled enormous calculations needed for tasks like gene editing
- Gene Editing
  - Investment in gene editing has surpassed \$5B
- Synthetic Biology

## Pharmaceuticals

- Artificial intelligence
  - AI is also extremely effective in discovering new drugs by simulating different combinations of chemicals
- Aging Population
  - Causes an increase in the number/complexity of illnesses
- Chronic Diseases
  - A byproduct of the aging population, chronic diseases are particularly expensive to treat

# Risks

**1**

## **Vast majority of revenues are derived from product sales for treatment of Cystic Fibrosis**

- Cases continue to go up, also diversification can be reached through its partnership with CRISPR

**2**

## **Trikafta patent challenges allowed beginning October 23, 2023**

- Only 3-5% of challenges succeed. General entry patent expires in 2037.
- Hoping to release a new CF drug by the end of this year (vanzacaftor)

**3**

## **Exa-cel may not receive approval**

- While this definitely poses a risk for the stocks growth, it is also what makes it a compelling buy (58-74% make it to market)
- Buying the stock before approval is where a large part of the return will be derived

**4**

## **High price tag on Exa-cel**

- Although it still may be difficult to convince buyers, they have a justified price in mind based off of the lifetime costs of these aggressive blood diseases

# Valuation

## Vertex Pharmaceuticals Inc. Discounted Cash Flow

(YE 31-Dec, USDm)

	2022A	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E
Revenue	8,930.9	10,574.2	12,604.4	14,948.9	17,520.1	20,323.3	23,412.4	26,760.4	30,373.0	34,473.4	39,127.3
% of Growth	17.9%	18.4%	19.2%	18.6%	17.2%	16.0%	15.2%	14.3%	13.5%	13.5%	13.5%
EBITDA	4,455.9	4,261.4	3,642.1	4,445.2	5,361.9	6,186.9	7,816.3	8,915.4	10,708.9	12,136.6	13,759.5
D&A		144.8	167.6	174.9	181.9	178.1	191.9	200.6	210.1	220.5	234.8
EBIT		4,116.6	3,474.5	4,270.3	5,180.0	6,008.8	7,624.5	8,714.8	10,498.7	11,916.0	13,524.7
Taxes		(926.1)	(1,103.6)	(1,189.7)	(1,340.0)	(1,482.0)	(1,607.7)	(1,750.7)	(1,864.6)	(1,859.7)	(1,982.4)
Capex		(226.5)	(229.4)	(242.0)	(255.1)	(251.1)	(268.9)	(281.5)	(293.8)	(305.8)	(322.3)
NWC Change		(73.6)	(130.0)	(160.6)	(162.5)	(258.8)	(267.1)	(191.0)	(188.1)	(175.7)	393.9
<b>FCF</b>		3,035.3	2,179.2	2,852.9	3,604.3	4,195.0	5,672.8	6,692.2	8,362.4	9,795.4	11,848.7
Factor		0.9	0.9	0.8	0.7	0.7	0.6	0.6	0.5	0.5	0.5
Discounted FCF		2,814.9	1,874.2	2,275.4	2,666.0	2,877.6	3,608.7	3,948.1	4,575.3	4,970.1	5,575.4

Sensitivity Table		WACC					
		6%	7%	8%	9%	10%	
PGR	1.0%	188,400.0	150,430.9	123,880.8	104,347.3	89,429.3	
	1.5%	205,680.6	161,091.9	130,915.1	109,217.7	92,926.0	
	2.0%	227,508.8	173,973.8	139,162.3	114,804.3	96,871.0	
	2.5%	255,951.6	189,851.6	148,965.5	121,277.7	101,356.5	
	3.0%	294,552.5	209,907.7	160,811.0	128,867.2	106,501.5	

### Assumptions

**Sales:** Modest increase in 2023 growth and more drastic increase in 2024 indicating initial approval and sale of Exa-cel

**COGS Margins:** Increase in 2023-2027 reflecting new technology and manufacturing needed for launches over the next 5 years



# Valuation cont.

Perpetuity Growth Method - Value per Share	
<b>WACC</b>	7.83%
<b>PGR</b>	2.00%
<b>Terminal value</b>	219,150.0
Discounted TV	103,120.6
PV of CF	35,185.8
<b>EV</b>	<b>138,306.4</b>
Net Debt	899.7
<b>Equity Value</b>	<b>137,406.7</b>
Shares Outstanding	257.6
<b>Implied Per Share Value</b>	<b>533.41</b>
Current Price (4/21/23)	331.09
<b>Estimated Upside</b>	<b>61.1%</b>

EBITDA Multiple Method - Value per Share	
2032 EBITDA	13,759.5
Terminal EBITDA Multiple	15.9x
Undiscounted Terminal Value	218,776.7
PV of CF	35,185.8
Discounted Terminal Value	102,945.0
<b>EV</b>	<b>138,130.7</b>
Net Debt	899.7
<b>Equity Value</b>	<b>137,231.0</b>
Shares Outstanding	257.60
<b>Implied Per Share Value</b>	<b>532.73</b>
Current Price (4/21/23)	331.09
<b>Estimated Upside</b>	<b>60.9%</b>



# Comparable Company Analysis

Company Name	TEV/Total Revenues LTM-Latest	TEV/EBITDA LTM- Latest	TEV/EBIT LTM-Latest	P/Diluted EPS Before Extra LTM-Latest
<b>Vertex Pharmaceuticals (NasdaqGS:VRTX)</b>	8.4x	16.5x	17.2x	25.8x
Biogen Inc. (NasdaqGS:BIIB)	4.3x	12.0x	14.4x	14.1x
Regeneron Pharmaceuticals, Inc. (NasdaqGS:REGN)	6.1x	13.9x	14.8x	21.0x
Gilead Sciences, Inc. (NasdaqGS:GILD)	4.6x	9.5x	11.4x	23.8x
Amgen Inc. (NasdaqGS:AMGN)	6.1x	11.7x	16.0x	20.1x
Incyte Corporation (NasdaqGS:INCY)	4.0x	19.9x	22.6x	49.1x
Neurocrine Biosciences, Inc. (NasdaqGS:NBIX)	6.3x	33.4x	37.6x	66.8x
United Therapeutics Corporation (NasdaqGS:UTHR)	3.8x	7.0x	7.4x	15.4x
AbbVie Inc. (NYSE:ABBV)	5.9x	10.9x	15.1x	24.5x
Arcturus Therapeutics Holdings Inc. (NasdaqGM:ARCT)	2.1x	24.5x	37.6x	79.1x

	Total Enterprise Value Multiples			
	LTM TEV/Total Revenues	LTM TEV/EBITDA	LTM TEV/EBIT	LTM P/Diluted EPS Before Extra
<b>High</b>	6.3x	33.4x	37.6x	79.1x
<b>Low</b>	2.1x	7.0x	7.4x	14.1x
<b>Mean</b>	4.8x	15.9x	19.7x	34.9x

Implied Price Per Share				
<b>High</b>	256.2	624.5	676.8	1,025.86
<b>Low</b>	112.1	162.1	164.9	182.73
<b>Mean</b>	204.5	317.1	372.6	452.23

# Football Field

## Valuation of VRTX



# Allocation

- **Duration recommended**
  - 5-8 years to capture all the upside of the 5 new projects launched over the next five years
- **Catalysts**
  - Growth factors should occur by 2024
- **Consensus**
  - Differ by magnitude
- **Signposts**
  - FDA Approval on Exa-cel
  - Age expansion offerings for current CF Treatments
  - Approval of the numerous phase III launches in pipeline
- **Pre-Mortem**
  - People are not able or willing to pay high price point of Exa-cel
  - New launches are not approved by the FDA
  - Patent expires on Trikafta

# Conclusion

We strongly recommend a long position in VRTX due to the implied price target of **\$533.41** which is **61.1%** upside from the current price of \$331.09.

Our analysis reveals that due to its **CRISPR Therapeutics partnership** and **dominant position among competitors**, this company will be driven by growth in its current markets while expanding into new ones.

# Appendix

# Bear Case

## Vertex Pharmaceuticals Inc. Discounted Cash Flow

(YE 31-Dec, USDm)

	2022A	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E
Revenue	8,930.9	10,368.8	11,944.8	13,664.9	15,468.6	17,417.7	19,490.4	21,634.3	23,906.0	26,416.1	29,189.8
% of Growth	17.9%	16.1%	15.2%	14.4%	13.2%	12.6%	11.9%	11.0%	10.5%	10.5%	10.5%
EBITDA	4,455.9	3,781.9	3,102.0	3,668.4	4,291.3	4,805.3	5,954.4	6,597.0	7,756.3	8,559.0	9,448.9
D&A		144.8	167.6	174.9	181.9	178.1	191.9	200.6	210.1	220.5	234.8
EBIT		3,637.1	2,934.4	3,493.6	4,109.4	4,627.2	5,762.5	6,396.4	7,546.1	8,338.5	9,214.0
Taxes		(926.1)	(1,103.6)	(1,189.7)	(1,340.0)	(1,482.0)	(1,607.7)	(1,750.7)	(1,864.6)	(1,859.7)	(1,982.4)
Capex		(226.5)	(229.4)	(242.0)	(255.1)	(251.1)	(268.9)	(281.5)	(293.8)	(305.8)	(322.3)
NWC Change		(73.6)	(130.0)	(160.6)	(162.5)	(258.8)	(267.1)	(191.0)	(188.1)	(175.7)	393.9
<b>FCF</b>		2,555.8	1,639.0	2,076.1	2,533.8	2,813.4	3,810.8	4,373.8	5,409.8	6,217.8	7,538.0
Factor		0.9	0.9	0.8	0.7	0.7	0.6	0.6	0.5	0.5	0.5
Discounted FCF		2,370.2	1,409.6	1,655.9	1,874.2	1,929.9	2,424.3	2,580.4	2,959.8	3,154.9	3,547.0

# Bear Case cont.

Perpetuity Growth Method - Value per Share	
<b>WACC</b>	7.83%
<b>PGR</b>	2.00%
<b>Terminal value</b>	139,420.8
Discounted TV	65,604.2
PV of CF	23,906.1
<b>EV</b>	<b>89,510.3</b>
Net Debt	899.7
<b>Equity Value</b>	<b>88,610.6</b>
Shares Outstanding	257.6
<b>Implied Per Share Value</b>	<b>343.99</b>
Current Price (4/21/23)	331.09
<b>Estimated Upside</b>	<b>3.9%</b>

EBITDA Multiple Method - Value per Share	
2032 EBITDA	9,448.9
Terminal EBITDA Multiple	15.9x
Undiscounted Terminal Value	150,236.8
PV of CF	23,906.1
Discounted Terminal Value	70,693.6
<b>EV</b>	<b>94,599.8</b>
Net Debt	899.7
<b>Equity Value</b>	<b>93,700.1</b>
Shares Outstanding	257.60
<b>Implied Per Share Value</b>	<b>363.74</b>
Current Price (4/21/23)	331.09
<b>Estimated Upside</b>	<b>9.9%</b>



# Bull Case

## Vertex Pharmaceuticals Inc. Discounted Cash Flow

(YE 31-Dec, USDm)

	2022A	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E
Revenue	8,930.9	10,663.5	12,817.5	15,329.8	18,119.8	21,200.1	24,634.6	28,403.6	32,522.2	37,237.9	42,637.4
% of Growth	17.9%	19.4%	20.2%	19.6%	18.2%	17.0%	16.2%	15.3%	14.5%	14.5%	14.5%
EBITDA	4,455.9	4,334.6	3,700.9	4,554.0	5,539.2	6,446.1	8,214.3	9,450.5	11,451.7	13,092.1	14,972.8
D&A		144.8	167.6	174.9	181.9	178.1	191.9	200.6	210.1	220.5	234.8
EBIT		4,189.8	3,533.3	4,379.1	5,357.3	6,268.0	8,022.5	9,249.9	11,241.6	12,871.6	14,738.0
Taxes		(926.1)	(1,103.6)	(1,189.7)	(1,340.0)	(1,482.0)	(1,607.7)	(1,750.7)	(1,864.6)	(1,859.7)	(1,982.4)
Capex		(226.5)	(229.4)	(242.0)	(255.1)	(251.1)	(268.9)	(281.5)	(293.8)	(305.8)	(322.3)
NWC Change		(73.6)	(130.0)	(160.6)	(162.5)	(258.8)	(267.1)	(191.0)	(188.1)	(175.7)	393.9
<b>FCF</b>		3,108.5	2,237.9	2,961.7	3,781.6	4,454.2	6,070.8	7,227.3	9,105.3	10,751.0	13,062.0
Factor		0.9	0.9	0.8	0.7	0.7	0.6	0.6	0.5	0.5	0.5
Discounted FCF		2,882.8	1,924.7	2,362.2	2,797.2	3,055.4	3,861.9	4,263.8	4,981.7	5,454.9	6,146.3



# Bull Case cont.

Perpetuity Growth Method - Value per Share	
<b>WACC</b>	7.83%
<b>PGR</b>	2.00%
<b>Terminal value</b>	241,590.7
Discounted TV	113,680.0
PV of CF	37,731.1
<b>EV</b>	<b>151,411.1</b>
Net Debt	899.7
<b>Equity Value</b>	<b>150,511.4</b>
Shares Outstanding	257.6
<b>Implied Per Share Value</b>	<b>584.28</b>
Current Price (4/21/23)	331.09
<b>Estimated Upside</b>	<b>76.5%</b>

EBITDA Multiple Method - Value per Share	
2032 EBITDA	14,972.8
Terminal EBITDA Multiple	15.9x
Undiscounted Terminal Value	238,068.1
PV of CF	37,731.1
Discounted Terminal Value	112,022.5
<b>EV</b>	<b>149,753.5</b>
Net Debt	899.7
<b>Equity Value</b>	<b>148,853.8</b>
Shares Outstanding	257.60
<b>Implied Per Share Value</b>	<b>577.85</b>
Current Price (4/21/23)	331.09
<b>Estimated Upside</b>	<b>74.5%</b>

# WACC Calculation

## WACC

Market Risk Premium ( $R_m - R_f$ )	8.16%
Multiplied by: VRTX Bottom-Up Beta	54.93%
Adjusted Market Risk Premium	4.48%
Add: Risk-Free Rate of Return ( $R_f$ )(1)	3.40%
<b>Cost of Equity</b>	<b>7.88%</b>
Multiplied by: VRTX $E/(D+P+E)$	98.95%
Cost of Equity Portion	7.80%
VRTX Cost of Debt ( $R_d$ ) - 10 Year Treasury Note	3.58%
Tax Rate (5 Year Average)	16.09%
<b>After-Tax Cost of Debt</b>	<b>3.00%</b>
Multiplied by: VRTX $D/(D+P+E)$	1.05%
Cost of Debt Portion	0.03%

<b>WACC</b>	<b>7.83%</b>
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# Additional Value Drivers

**1**

## **Strategic acquisitions of Semma (2019, \$950 million) and Viacyte (2022, \$320 million)**

- a) The addition of these two companies has allowed them to establish a presence in type 1 diabetes
- b) Currently developing a type 1 diabetes stem cell based therapy, in early trials

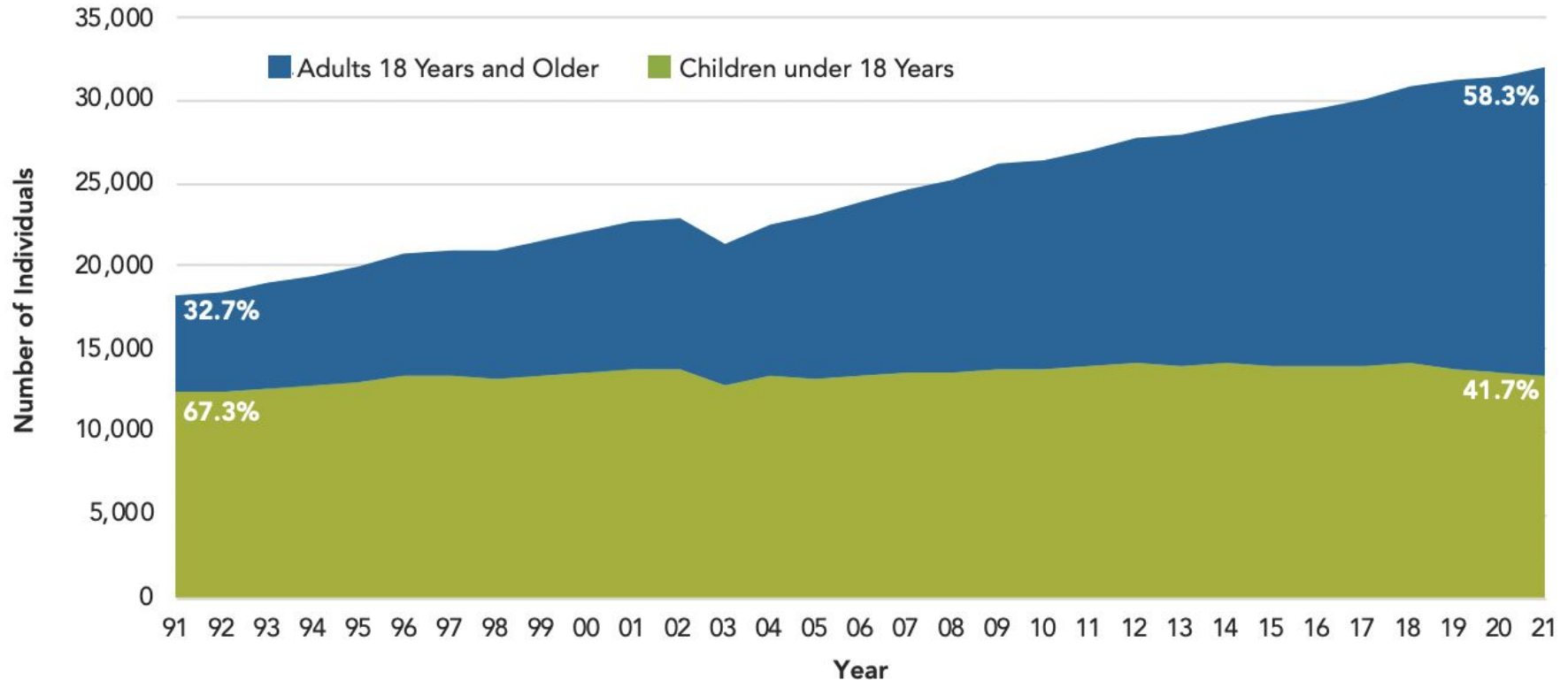
**2**

## **New therapies for kidney disease and pain management**

- a) Acute pain management treatment with a phase III trial expected for completion in late 2023/early 2024 (fast track and breakthrough designations granted)
- b) Therapy for APOL1 mediated kidney disease (phase II)

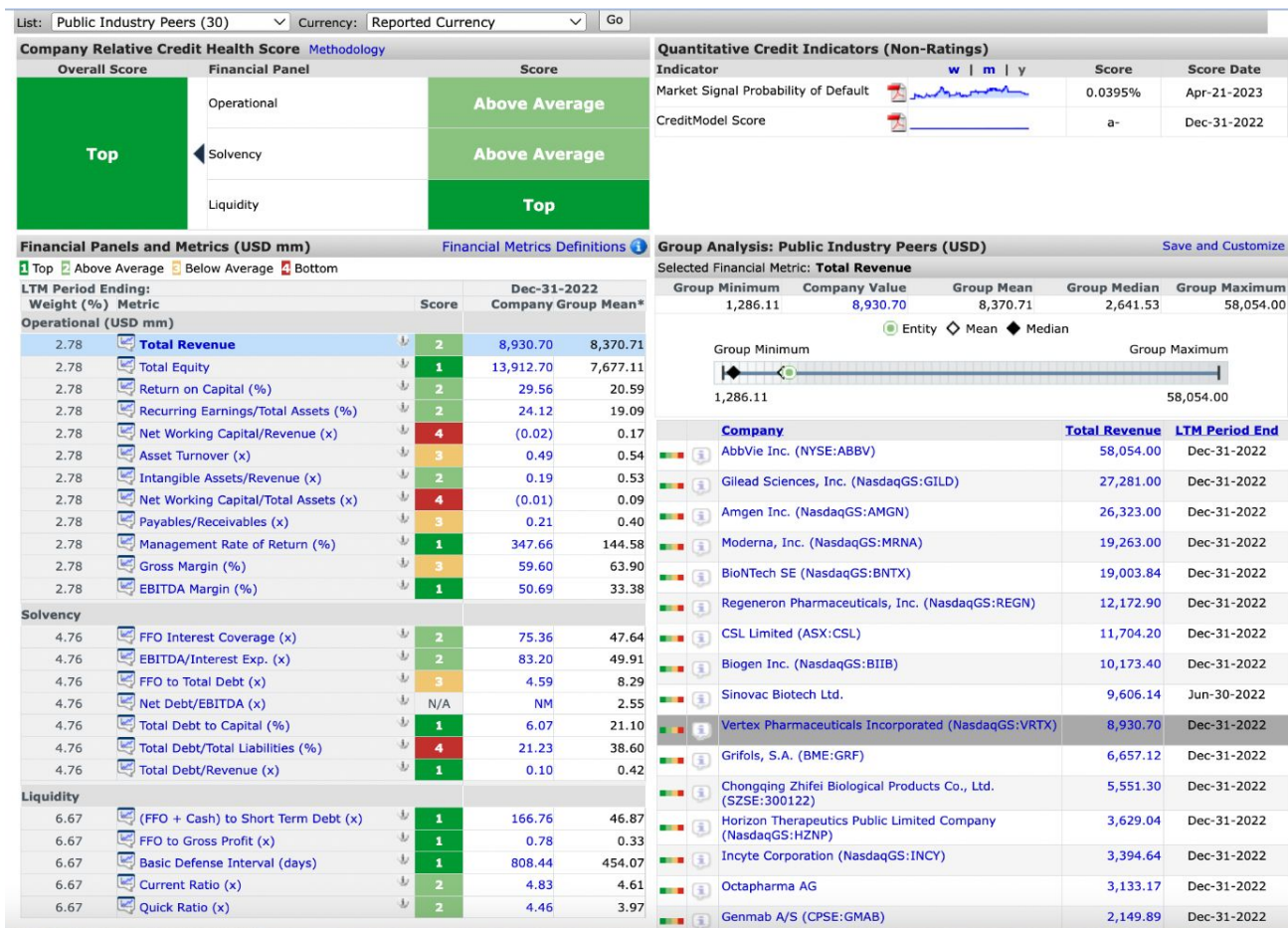
# CF Rates

Number of Children and Adults with CF, 1991–2021



*The decrease in the number of individuals reported in 2003 is due to a delay in obtaining informed consent forms before the close of the calendar year at some CF care centers.*

# Debt and Credit Rating



**Total Debt: \$899.7 MM**  
**Debt to EBITDA: 0.2x**