

## US Biopharmaceuticals

## Tuesday conference takeaways: TVTX, NTLA, EDIT

Industry Overview

## Traverse prepared for strong year with Filspari in IgAN

[After the recent update which included preliminary sales for 4Q \(see report\)](#), Traverse provided an encouraging corporate update at a healthcare conference, with the focus surrounding the process towards a full approval of Filspari in IgAN. [As one of our top picks for 2024 \(see report\)](#), we look for a successful filing and acceptance of the sNDA for Filspari in IgAN, and we model \$105M in 2024 Filspari sales and \$1.1B in risk-adj. peak sales (75% PoS). Building upon the [presentation during our December bus tour \(see our takeaways\)](#), the company talked through the positive FDA feedback as Traverse [remains on track for a 1Q24 sNDA filing \(see report\)](#) and 4Q23 sales growth including a positive increase in new physician prescribers. We look for the sales growth to continue into 2024 as the company reaches a full year of approval and also details surrounding a potential path forward for sparsentan in FSGS during the year. The company also mentioned the opportunity for discussions with the FDA surrounding potentially removing the REMS program, and reviewed the timeline for CHMP opinion (1Q24) and EMA decision (2Q24) for the full approval of Filspari in the EU with partner Vifor Pharma. Looking at pegtibatase, the company reviewed the ongoing phase 3 HARMONY study, which is set to have topline data in 2026, building upon positive data from the COMPOSE study. Given the ongoing launch of Filspari in IgAN and encouraging late stage progress of pegtibatase development, we maintain Buy, PO \$23.

## Intellia reviews positive pivotal stage progress

[After providing a corporate update last week \(see report\)](#), Intellia reviewed the strategic plan for 2024 -2026, which is led by multiple pivotal trials evaluating gene editing treatments, NTLA-2001 in ATTR-CM (dosing 1Q24) and ATTR-PN (planning ongoing) and NTLA-2002 in HAE (initiating 2H24; BLA 2026). While we look for future clinical readouts including updates from the ongoing early stage trials [to build upon positive clinical data \(see report\)](#), we think Intellia is in a solid position for 2024 across its pipeline including bringing the early stage NTLA-3001 into the clinic with initial dosing in 2024. Given the ongoing clinical development including movement towards multiple pivotal stage trials, we maintain Buy, PO \$80.

## Editas looks to deepen reni-cel dataset

Editas announced a detailed corporate update with the focus surrounding the development of reni-cel (EDIT-301) in the RUBY (sickle cell disease) and EDITHAL (thalassemia) trials. [After positive data at ASH \(see report\)](#), we look for the mid-2024 and YE24 clinical updates in sickle cell to show sustained durability, and signs of hemoglobin normalization across patients. The company also highlighted early stage research on in vivo gene editing with an initial focus in hematopoietic stem cells, and we look for preclinical data to show proof of concept. Beyond clinical applications, Editas also noted its strong IP profile and recent license agreements with Vertex and Vor Bio. Maintain Neutral, PO \$15.

See our updated catalyst calendar below (Exhibit 1):

09 January 2024

Equity  
United States  
Biopharmaceuticals

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TVTX: Traverse Therapeutics

NTLA: Intellia

EDIT: Editas

IgAN: IgA nephropathy

FSGS: focal segmental glomerulosclerosis

ATTR-CM: transthyretin amyloidosis cardiomyopathy

ATTR-PN: transthyretin amyloidosis polyneuropathy

CHMP: Committee for Medicinal Products for Human Use

BLA: biologics license application

HAE: hereditary angioedema

sNDA: supplemental new drug application

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Timestamp: 09 January 2024 10:14PM EST

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**Exhibit 1: Catalyst Calendar**

Travere remains on track to file an sNDA for the full approval of Filspari in IgAN.

Company	Asset	Indication	Event	Timing	Importance
Editas	EDIT-301	SCD	RUBY clinical update	mid-24	High
Editas	EDIT-301	SCD	RUBY clinical update	YE24	High
Intellia	NTLA-2001	ATTR-CM	Dose patient in phase 3 trial	1Q24	Moderate
Intellia	NTLA-2001	ATTR	Present updated phase 1 data	2024	High
Intellia	NTLA-2002	HAE	Initiate a global pivotal phase 3 trial	2H24	Moderate
Intellia	NTLA-2002	HAE	Present phase 1/2 data	2024	High
Intellia	NTLA-3001	AATD lung disease	Dose patient in phase 1 trial	2024	Low
Travere	Sparsentan	IgAN	CHMP opinion	1Q24	High
Travere	Sparsentan	IgAN	Potential EMA decision	2Q24	High
Travere	Sparsentan	IgAN	sNDA filing	1Q24	High
Travere	Sparsentan	IgAN	Filspari + SGLT2i combo data and first line Filspari data	2024	Moderate
Travere	pegibatinase HCU		HARMONY topline data	2026	High

Source: BofA Global Research, company reports

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**Exhibit 2: Stocks mentioned**

Prices and ratings for stocks mentioned

BofA Ticker	Bloomberg ticker	Company name	Price	Rating
EDIT	EDIT US	Editas	US\$ 9.22	C-2-9
NTLA	NTLA US	Intellia	US\$ 29.29	C-1-9
TVTX	TVTX US	Travere Therapeutics	US\$ 9.8	C-1-9

Source: BofA Global Research

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**Price objective basis & risk****Editas Medicine (EDIT)**

Our \$15 share price objective is based on a probability adjusted (50%) net present value (NPV) analysis of, EDIT-301 in SCD/TDT (\$6/share), \$1/sh on royalties, \$2/share in platform value, and net cash (\$6/share). We use a weighted-average cost of capital (WACC) of 15%, similar to other early-stage companies in our coverage universe and a -2% terminal growth rate.

Upside risks: higher-than-anticipated uptake of approved products, better-than-expected clinical trial results.

Downside risks: unexplained safety signals, clinical trial failures, and strong data from competitors.

**Intellia Therapeutics (NTLA)**

Our \$80 share price objective is based on a probability adjusted (60-75%) net present value (NPV) analysis of NTLA-2001 in ATTR (\$50/share), NTLA-2002 in HAE (\$12/share), platform value (\$8/share) and net cash (\$10/share). We use a weighted average cost of capital (WACC) of 15% similar to other clinical-stage companies in our coverage universe and a -2% terminal growth rate.

Downside risks: unexpected safety signals, clinical trial failures, and strong data from competitors.

**Travere Therapeutics Inc (TVTX)**

Our \$23 PO is based on a sum-of-the-parts NPV analysis. Using a 13% WACC, we model \$2/share in bile acid portfolio milestones, \$3/share for Thiola, \$13/share for sparsentan, \$1/share for pegtibatase, and \$3/share net cash.

Risks are 1) regulatory risk, 2) competitive entrants, 3) lower-than-expected sparsentan uptake, 4) insurance or pricing concerns

## Analyst Certification

I, Greg Harrison, CFA, hereby certify that the views expressed in this research report accurately reflect my personal views about the subject securities and issuers. I also certify that no part of my compensation was, is, or will be, directly or indirectly, related to the specific recommendations or view expressed in this research report.

## US - Biopharmaceuticals Coverage Cluster

Investment rating	Company	BofA Ticker	Bloomberg symbol	Analyst
<b>BUY</b>	89bio, Inc	ETNB	ETNB US	Geoff Meacham
	Acumen Pharma	ABOS	ABOS US	Geoff Meacham
	Agios Pharmaceuticals	AGIO	AGIO US	Greg Harrison, CFA
	Amylyx Pharmaceuticals	AMLX	AMLX US	Geoff Meacham
	BioMarin	BMRN	BMRN US	Geoff Meacham
	BioXcel Therapeutics	BTAI	BTAI US	Greg Harrison, CFA
	BridgeBio Pharma	BBIO	BBIO US	Greg Harrison, CFA
	Caribou	CRBU	CRBU US	Geoff Meacham
	CRISPR Therapeutics	CRSP	CRSP US	Geoff Meacham
	Eli Lilly and Company	LLY	LLY US	Geoff Meacham
	Gilead Sciences Inc.	GILD	GILD US	Geoff Meacham
	HUTCHMED	HCM	HCM US	Alec W. Stranahan
	Immatics	IMTX	IMTX US	Alec W. Stranahan
	Insmid Incorporated	INSM	INSM US	Jason Zemansky
	Intellia Therapeutics	NTLA	NTLA US	Greg Harrison, CFA
	Janux Therapeutics	JANX	JANX US	Geoff Meacham
	Keros	KROS	KROS US	Greg Harrison, CFA
	Kiniksa Pharmaceuticals, Ltd.	KNSA	KNSA US	Geoff Meacham
	Krystal Biotech	KRYS	KRYS US	Alec W. Stranahan
	Kura Oncology	KURA	KURA US	Jason Zemansky
	Liquidia Corporation	LQDA	LQDA US	Greg Harrison, CFA
	Lyell Immunopharma	LYEL	LYEL US	Geoff Meacham
	MeiraGTx	MGTX	MGTX US	Alec W. Stranahan
	Merck & Co.	MRK	MRK US	Geoff Meacham
	Mineralys Therapeutics	MLYS	MLYS US	Greg Harrison, CFA
	Neumora Therapeutics	NMRA	NMRA US	Geoff Meacham
	Rani Therapeutics	RANI	RANI US	Geoff Meacham
	Regenxbio, Inc.	RGNX	RGNX US	Alec W. Stranahan
	Revolution Medicines	RVMD	RVMD US	Alec W. Stranahan
	Rocket Pharmaceuticals, Inc.	RCKT	RCKT US	Greg Harrison, CFA
	Royalty Pharma	RPRX	RPRX US	Geoff Meacham
	Sana Biotechnology	SANA	SANA US	Geoff Meacham
	SpringWorks	SWTX	SWTX US	Alec W. Stranahan
	Syndax Pharmaceuticals	SNDX	SNDX US	Jason Zemansky
	Traverse Therapeutics Inc	TVTX	TVTX US	Greg Harrison, CFA
	Turnstone Biologics	TSBX	TSBX US	Geoff Meacham
	Vertex Pharmaceuticals Inc.	VRTX	VRTX US	Geoff Meacham
	Werewolf Therapeutics	HOWL	HOWL US	Jason Zemansky
	Xencor	XNCR	XNCR US	Alec W. Stranahan
<b>NEUTRAL</b>	AbbVie	ABBV	ABBV US	Geoff Meacham
	Alector, Inc	ALEC	ALEC US	Greg Harrison, CFA
	Amgen Inc.	AMGN	AMGN US	Geoff Meacham
	Arcus Biosciences	RCUS	RCUS US	Jason Zemansky
	Beam Therapeutics	BEAM	BEAM US	Greg Harrison, CFA
	Biogen Inc.	BIIB	BIIB US	Geoff Meacham
	Bristol-Myers Squibb	BMJ	BMJ US	Geoff Meacham
	Cytokinetics, Incorporated	CYTK	CYTK US	Jason Zemansky
	Editas Medicine	EDIT	EDIT US	Greg Harrison, CFA
	Erasca	ERAS	ERAS US	Alec W. Stranahan
	Esperion	ESPR	ESPR US	Jason Zemansky
	Exscientia	EXAI	EXAI US	Alec W. Stranahan
	IGM Biosciences	IGMS	IGMS US	Greg Harrison, CFA
	Johnson & Johnson	JNJ	JNJ US	Geoff Meacham
	Kymira Therapeutics	KYMR	KYMR US	Geoff Meacham
	Moderna	MRNA	MRNA US	Geoff Meacham
	Pfizer	PFE	PFE US	Geoff Meacham
	Recursion Pharmaceuticals, Inc.	RXR	RXR US	Alec W. Stranahan
	Tyra Biosciences	TYRA	TYRA US	Greg Harrison, CFA
	Vir	VIR	VIR US	Geoff Meacham
	Y-mAbs Therapeutics, Inc	YMAB	YMAB US	Alec W. Stranahan
<b>UNDERPERFORM</b>	AlloVir, Inc.	ALVR	ALVR US	Jason Zemansky

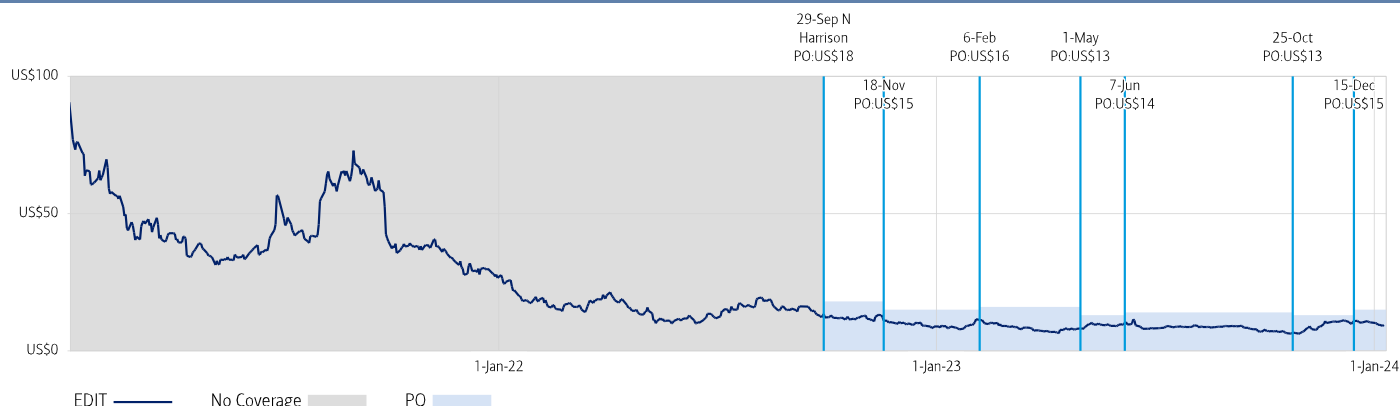
## US - Biopharmaceuticals Coverage Cluster

Investment rating	Company	BofA Ticker	Bloomberg symbol	Analyst
	CureVac	CVAC	CVAC US	Geoff Meacham
	Day One Biopharmaceuticals	DAWN	DAWN US	Alec W. Stranahan
	LianBio	LIAN	LIAN US	Geoff Meacham
	Novavax	NVAX	NVAX US	Alec W. Stranahan
	Regeneron Pharmaceuticals Inc.	REGN	REGN US	Geoff Meacham
	Reneo Pharmaceuticals	RPHM	RPHM US	Jason Zemansky
	TG Therapeutics	TGTX	TGTX US	Alec W. Stranahan
	United Therapeutics Corporation	UTHR	UTHR US	Greg Harrison, CFA

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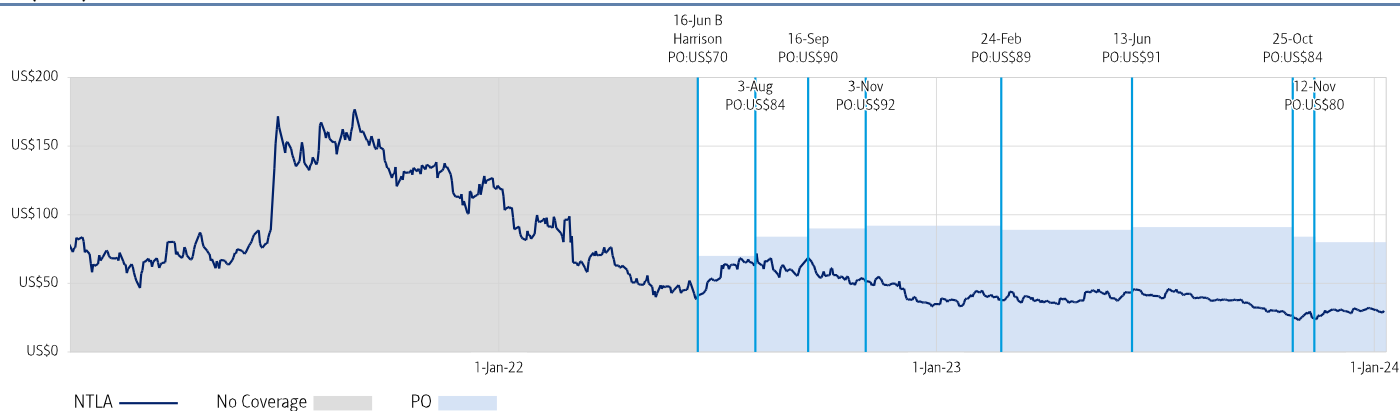
## Important Disclosures

## Editas (EDIT) Price Chart



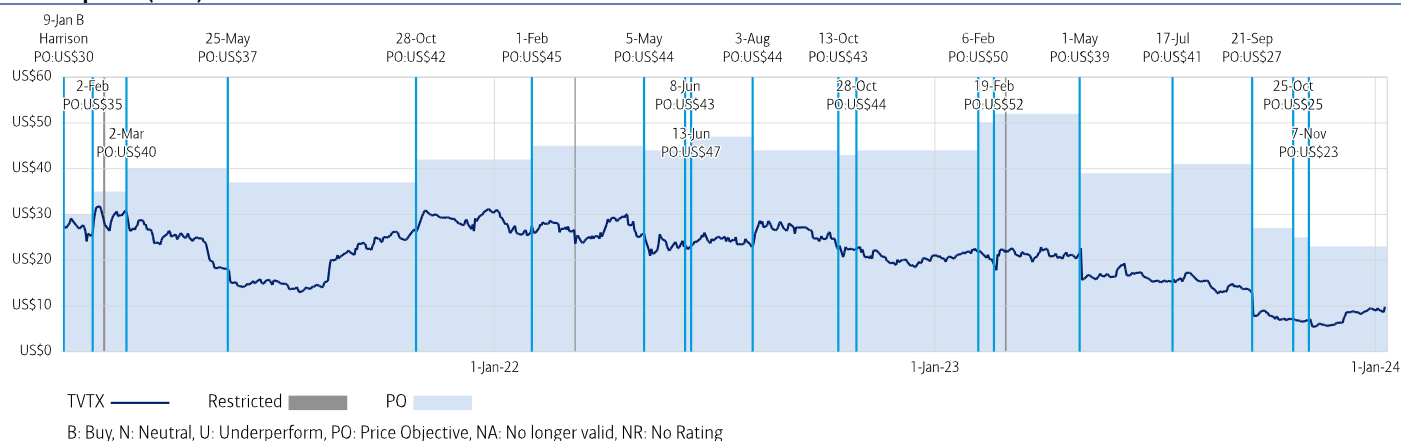
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## Intellia (NTLA) Price Chart



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## Traverse Therapeutics (TVTX) Price Chart



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## Equity Investment Rating Distribution: Health Care Group (as of 31 Dec 2023)

Coverage Universe	Count	Percent	Inv. Banking Relationships <sup>R1</sup>	Count	Percent
Buy	234	60.94%	Buy	115	49.15%
Hold	80	20.83%	Hold	36	45.00%
Sell	70	18.23%	Sell	29	41.43%

## Equity Investment Rating Distribution: Global Group (as of 31 Dec 2023)

Coverage Universe	Count	Percent	Inv. Banking Relationships <sup>R1</sup>	Count	Percent
Buy	1895	53.62%	Buy	1083	57.15%
Hold	832	23.54%	Hold	454	54.57%
Sell	807	22.84%	Sell	383	47.46%

<sup>R1</sup> Issuers that were investment banking clients of BofA Securities or one of its affiliates within the past 12 months. For purposes of this Investment Rating Distribution, the coverage universe includes only stocks. A stock rated Neutral is included as a Hold, and a stock rated Underperform is included as a Sell.

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Investment rating	Total return expectation (within 12-month period of date of initial rating)	Ratings dispersion guidelines for coverage cluster <sup>R2</sup>
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Neutral	≥ 0%	≤ 30%
Underperform	N/A	≥ 20%

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