

US Biopharmaceuticals

SMid biotech 4Q model updates: RCKT, KROS, BEAM

Price Objective Change

Clinical catalysts remain on track across the board

We update our model for 4Q updates from Rocket, Beam and Keros. Our ratings remain the same and we detail estimate and PO changes in Exhibit 2.

Rocket ready to launch with June 30th PDUFA in place

Rocket announced a 4Q23 update highlighting progress of late stage clinical development including the ongoing pivotal Danon program and a [June 30th PDUFA set for Kresladi in LAD-1 \(see report\)](#). We look for further details surrounding launch preparations for Rocket's initial commercial product, which, while a small population, we think can provide a scalable foundation for commercial gene therapy products in larger indications. Along with a MAA/BLA filing expected for the FA program in 1H24, Rocket also plans to make progress across its pipeline with a pivotal trials ongoing in PKD and Danon (interim data 2024), a phase 1 trial ongoing in PKP2-ACM and IND-enabling studies ongoing for the BAG3-DCM program. Rocket has \$407.5M in cash and cash equivalents with a cash runway into 2026. We update our model for 4Q23 reporting and alter our OpEx assumptions for upcoming years, resulting in a new PO of \$39 (from \$37). We now model 2024 and 2025 EPS of -\$3.26 (from -\$3.21) and -\$2.87 (from -\$2.42), respectively. Reiterate Buy, PO \$39 (from \$37)

Beam brightens ahead of derisking clinical data

Beam provided a positive 4Q23 update with a focus on planned clinical progress in 2024 including BEACON enrollment progress (expansion cohort initiation 1H24) and anticipated interim BEACON data from multiple patients treated with BEAM-101 (2H24). We look for the company to continue to collect and present derisking data with multiple programs approaching the clinic, BEAM-302 in GSD1a (phase 1 initiation 1H24) and BEAM-301 in AATD (IND 1H24) and look for clinical progress to add to our model. We also see encouraging steps forward from ESCAPE research programs as the company plans to initiate phase 1 enabling preclinical studies (2024). With the [recent deal with Lilly \(see report\)](#) highlighting the value seen for base editing products, we look for future updates from Beam's multiple collaboration deals. Beam has \$1.2B in cash and cash equivalents and a cash runway into 2027. Overall we believe Beam continues to execute [on the restructuring plans \(see report\)](#) and we update our model for 4Q23 reporting, update our OpEx assumptions, and increase our PoS for BEAM-101 to 40% (from 30%), resulting in a new PO of \$42 (from \$35). We now model 2024 and 2025 EPS of -\$5.32 (from -\$5.62) and -\$5.47 (from -\$5.60), respectively. Reiterate Neutral, new PO \$42 (from \$35).

See our Keros takeaways and catalyst calendar below (Exhibit 1):

01 March 2024

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United States
Biopharmaceuticals

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MAA/BLA: Marketing Authorization Application/ Biologics License Application

FA: Fanconi anemia

LAD-1: leukocyte adhesion deficiency type 1

PKD: pyruvate kinase deficiency

IND: Investigational New Drug application

PKP2-ACM: PKP2 arrhythmogenic cardiomyopathy

BAG3-DCM: BAG3 associated dilated cardiomyopathy

GSD1a: glycogen storage disease type 1a

AATD: alpha-1 antitrypsin deficiency

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Refer to important disclosures on page 5 to 8. Analyst Certification on page 3. Price Objective Basis/Risk on page 3.

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Timestamp: 01 March 2024 07:00AM EST

Keros set to fuel late stage development with 2024 data

Keros announced a 4Q23 update with a focus on the ongoing clinical pipeline programs, which remain on track for upcoming catalysts, and a financial overview. [After positive data at ASH 2023 \(see report\)](#), we look for encouraging data from both KER-050 programs in myelodysplastic syndrome (MDS) and myelofibrosis (MF) with multiple updates expected in 2024 (2Q/4Q) and future details surrounding engagement with regulators on phase 3 development in MDS (1H24). Beyond KER-050, we also look for encouraging progress from the KER-012 program including an enrollment update of the TROPOS trial in pulmonary arterial hypertension (1H24) and initial chronic heart failure data (2H24). With a phase 1 healthy volunteer clinical trial evaluating KER-065 ongoing, we also anticipate proof of concept data to support further development in obesity and neuromuscular indications. Keros has \$331M in cash and cash equivalents as of Dec 31, 2023 and with \$151M from the January offering of common stock, Keros has a cash runway into 2027. We update our model for 4Q23 reporting and add \$10/sh in platform value based on the muscle and obesity programs, resulting in a new PO of \$78 (from \$66). We now model 2024 and 2025 EPS of -\$4.41 (from -\$5.26) and -\$5.72 (from -\$4.37), respectively. Reiterate Buy, new PO \$78 (from \$66).

Exhibit 1: Catalyst Calendar

The PDUFA for the approval of RP-L201 is June 30, 2024.

Company	Asset	Indication	Event	Timing	Importance
Beam	BEAM-101	SCD	BEACON trial readout	2H24	High
Beam	BEAM-101	SCD	Expansion cohort initiation	1H24	Moderate
Beam	BEAM-201	T-ALL/T-LL	Initial data	2H24	High
Beam	BEAM-301	GSD1a	Regulatory filing	1H24	Low
Beam	BEAM-302	AATD	Trial initiation	1H24	Moderate
Keros	KER-050	MDS	Complete phase 2 TD enrollment	1H24	Low
Keros	KER-012	Chronic heart failure	Initial data from phase 2 trial	2H24	High
Keros	KER-012	PAH	Update on enrollment of TROPOS	1H24	Moderate
Keros	KER-050	MDS	Additional data from Ph2 part 2 MDS trial	2Q and 4Q24	High
Keros	KER-050	MF	Additional data from Ph2 MF trial	2Q and 4Q24	High
Keros	KER-065	Obesity/ neuromuscular	Phase 1 proof of concept data	1Q25	High
Rocket	RP-L201	LAD-1	PDUFA	30-Jun-24	High
Rocket	RP-A501	Danon disease	Pivotal trial interim update	2024	High
Rocket	RP-L102	Fanconi anemia	BLA-MAA filing	1H24	High
Rocket	BAG3-association	DCM BAG3-DCM	IND filing	2024	Low

Source: BofA Global Research, company reports

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Exhibit 2: PO and estimate changes

We detail PO and estimate changes below.

Company	Previous PO	Current PO	Previous 2024 EPS	Current 2024 EPS	Previous 2025 EPS	Current 2025 EPS
BEAM	\$35	\$42	(\$5.62)	(\$5.32)	(\$5.60)	(\$5.47)
KROS	\$66	\$78	(\$5.26)	(\$4.41)	(\$4.37)	(\$5.72)
RCKT	\$37	\$39	(\$3.21)	(\$3.26)	(\$2.42)	(\$2.87)

Source: BofA Global Research

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Exhibit 3: Stocks mentioned

Prices and ratings for stocks mentioned in this report

BofA Ticker	Bloomberg ticker	Company name	Price	Rating
BEAM	BEAM US	Beam Therapeutics	US\$ 39.49	C-2-9
KROS	KROS US	Keros	US\$ 67.50	C-1-9
RCKT	RCKT US	Rocket Pharma	US\$ 29.03	C-1-9

Source: BofA Global Research

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Price objective basis & risk

Beam Therapeutics (BEAM)

Our \$42/share price objective is based on a probability adjusted (30%) NPV analysis of its primary program under development. We use a WACC of 15%, similar to other early-stage companies in our coverage universe and a -2% terminal growth rate.

Downside risks: 1) failure of early clinical trials, 2) dangerous safety signals, 3) superior competitor data, 4) soft market uptake.

Keros (KROS)

Our \$78/share price objective is based on a probability adjusted (20%-50%) net present value (NPV) analysis of its program under development. We use a weighted-average cost of capital (WACC) of 15%, similar to other early-stage companies in our coverage universe, and a terminal growth rate of -5%. Given these assumptions we estimate a value of \$36/sh for KER-050 in MDS (previously of \$38/sh), \$3/sh for KER-050 in MF, \$6/sh in KER-050 royalties, \$9/sh in KER-012 (previously \$10/sh), \$12/sh in platform value, and \$12/sh in net cash (previously \$9/sh).

Upside risks are better than expect data in clinical trials including MDS/MF patients treated with KER-050 which could lead to higher than anticipated usage if approved.

Downside risks are unexpected safety signals, clinical trial failures, and competitors releasing stronger data.

Rocket Pharmaceuticals, Inc. (RCKT)

Our \$39/share price objective is based on a probability-adjusted (35%-90%) net present value (NPV) analysis of its four programs under development. We use a weighted-average cost of capital (WACC) of 15%, similar to other early-stage companies in our coverage universe, and terminal growth rate of -2%. Given these assumptions, we estimate a value of \$9/share for RP-L102 (Fanconi anemia), \$23/share for RP-A501 (Danon disease) (previously \$21), \$2/share for RP-L201 (LAD-1), \$1/share for RP-L301 (PKD), and \$4/share in net cash.

Risks: 1) failure of early clinical trials, 2) emergence of unacceptable safety signals, 3) shorter efficacy duration than expected, and 4) commercialization failures.

Analyst Certification

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US - Biopharmaceuticals Coverage Cluster

Investment rating	Company	BofA Ticker	Bloomberg symbol	Analyst
BUY				
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	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
	Insmmed Incorporated	INSM	INSM US	Jason Zemansky
	Intellia Therapeutics	NTLA	NTLA US	Greg Harrison, CFA
	Janux Therapeutics	JANX	JANX US	Alec W. Stranahan
	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
NEUTRAL				
	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	Alec W. Stranahan
	Y-mAbs Therapeutics, Inc	YMAB	YMAB US	Alec W. Stranahan
UNDERPERFORM				
	AlloVir, Inc.	ALVR	ALVR US	Jason Zemansky

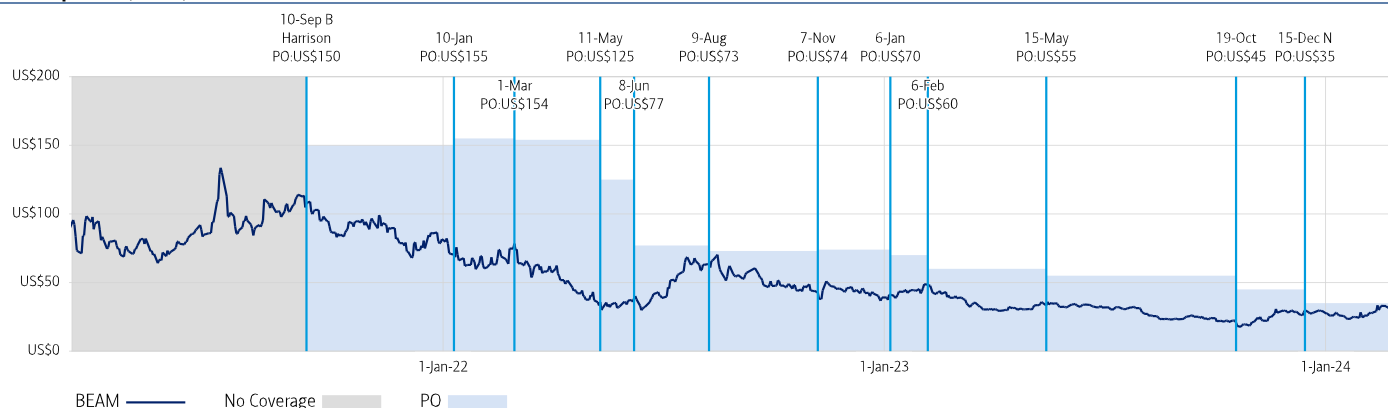
US - Biopharmaceuticals Coverage Cluster

Investment rating	Company	BofA Ticker	Bloomberg symbol	Analyst
	CureVac	CVAC	CVAC US	Geoff Meacham
	Day One Biopharmaceuticals	DAWN	DAWN US	Alec W. Stranahan
	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

Disclosures

Important Disclosures

Beam Therapeutics (BEAM) Price Chart



B: Buy, N: Neutral, U: Underperform, PO: Price Objective, NA: No longer valid, NR: No Rating

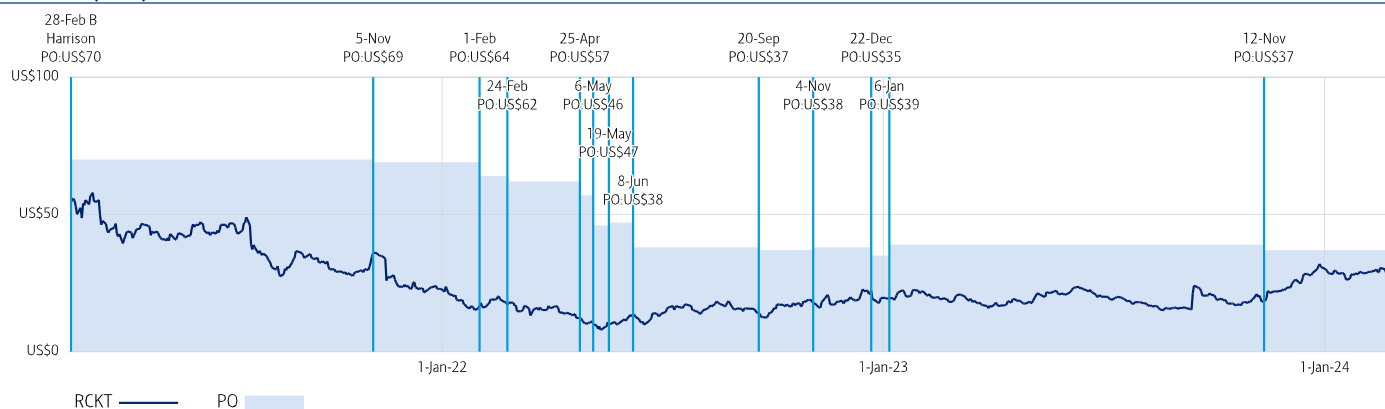
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Keros (KROS) Price Chart



B: Buy, N: Neutral, U: Underperform, PO: Price Objective, NA: No longer valid, NR: No Rating

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Rocket Pharma (RCKT) Price Chart

B: Buy, N: Neutral, U: Underperform, PO: Price Objective, NA: No longer valid, NR: No Rating

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

Coverage Universe	Count	Percent	Inv. Banking Relationships ^{R1}	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 ^{R1}	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%

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Underperform	N/A	≥ 20%

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