

January 24, 2024

HEALTHCARE/BIO TECHNOLOGY

Key Takeaways from Sachs Neuroscience Innovation Forum

Strategic Activity Drives Investor Optimism

SUMMARY

We joined [Sachs Associates 7th Annual Neuroscience Innovation Forum](#) featuring 367 participants from 24 countries and came away incrementally more optimistic about opportunities for investors this year. We moderated a panel discussion on New Modalities for Rare Neurological Diseases covering diverse topics including: 1) continued progress with small molecules; 2) impact of IRA on novel modalities and rare diseases; 3) FDA approvals despite failed clinical trials; 4) FDA WARP program promoting gene therapies; 5) impact of AI on discovery and development; 6) optimizing clinical trial design while minimizing risk; and 7) business development, partnering, and financing strategies. Our discussion and others resonated favorably with investors and corporate executives. We see strategic interest in neuroscience from large partners as a clear signal of innovation at attractive valuations. We're especially encouraged by recent deals from large pharma such as ABBV and BMV which expand the neuroscience landscape beyond traditional players like BIIB.

KEY POINTS

- Our panel and others discussed use of biomarkers to identify patients most likely to respond to therapy and track their progress during clinical trials. The concept of “Goldilocks” patients sick enough to demonstrate a clinical benefit but not too sick for therapeutic intervention was addressed. Ultimately, the goal is to design a study with an enriched population that optimizes clinical outcomes with the greatest probability of success (Exhibit 1).
- Our panel suggested a strategy of going to market first in patients with the most robust clinical signal. The specific recommendation is to establish a foothold in a small patient population where your therapy can demonstrate the most significant clinical benefit to support reimbursement in that focused group, which is least likely to get payor pushback because otherwise, you may attempt to shut down the launch in order to protect the budget (Exhibit 2).
- We evaluated consensus estimates for an illustrative sample of recently launched drugs for rare neurological diseases, and found they approach \$10B in annual revenues by the end of this decade, thus providing ample incentive for investors and corporations who pursue research in these areas. These dynamics contribute to deal activity in the space such as BIIB's acquisition of RETA (Exhibit 3).
- [Biotech/pharma M&A activity in 2023](#) was notably higher than 2022 based on total transaction value of \$155B vs. \$90B. Biotech/pharma deal number in 2019-23 by therapeutic area shows cancer remains among the largest therapeutic targets of M&A ranked by deal number, with increasing numbers of metabolic, rare disease, and CNS deals in 2023 vs. prior years (Exhibits 4-5).
- Biotech/pharma transaction value in 2019-23 by therapeutic area shows cancer was the largest M&A target area by transaction value in 2023. CNS continues to increase over time, and we expect continued strategic interest in CNS assets in 2024 (Exhibit 6).

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For analyst certification and important disclosures, see the Disclosure Appendix.

Exhibit 1: We joined Sachs Associates 7th Annual Neuroscience Innovation Forum where we co-moderated a panel discussion in collaboration with Joel Sandler from Lumanity.

Exploring New Modalities for Rare & Orphan Neurological Diseases
Panel Discussion - 11:00am to 12:00pm

SACHS ASSOCIATES **7th ANNUAL NEUROSCIENCE INNOVATION FORUM**
for Business Development, Licensing & Investment
7th January 2024 | Marines' Memorial Club, SF | USA

Panelists:
Andrea Malizia, CEO, IAMA Therapeutics S.r.l.
Arun Mistry, CMO, Minoryx Therapeutics S.L.
Dirk Thye, CEO & CMO, Quince Therapeutics
Ilise Lombardo, CEO, Noema Pharma AG
Jesús Martin-García, CEO, GeNeuro SA
Marine Beurdeley, Senior Director, Global Business Development, Ipsen

Co-Chaired by:
Joel Sandler, Principal
Jay Olson, Managing Director

Lumanity **OPPENHEIMER**

Source: Sachs Associates, Oppenheimer Research

Exhibit 2: Our discussion panel covered a diverse range of topics which gave us incremental optimism toward opportunities for neuroscience investors in 2024.

Exploring New Modalities for Rare & Orphan Neurological Diseases

New therapeutic modalities for CNS diseases

- Transport vehicles, brain shuttles
- RNAi based therapies, siRNA, ASOs
- Gene therapies, novel capsids
- Gene editing, cell therapies

Rare and orphan CNS diseases

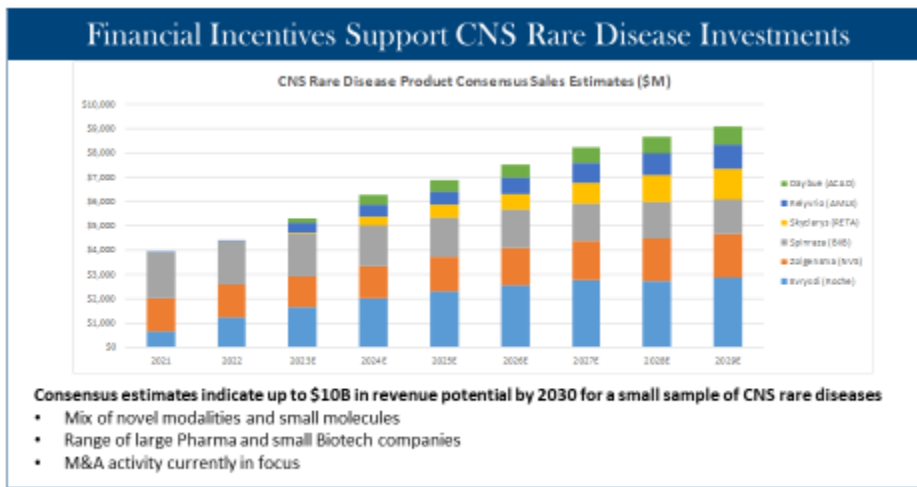
- Biomarkers for patient selection and approval
- Natural history studies, single arm clinical trials
- Symptomatic treatments vs disease modifying
- Wearable devices for clinical trials

Macro factors

- Continued innovation and progress with small molecules
- Impact of IRA on novel modalities and rare diseases
- FDA approvals despite failed clinical trials
- FDA WARP program promoting gene therapies
- Impact of AI on discovery and development
- Optimizing clinical trial design, minimizing risk
- Business development, partnering, financing strategies

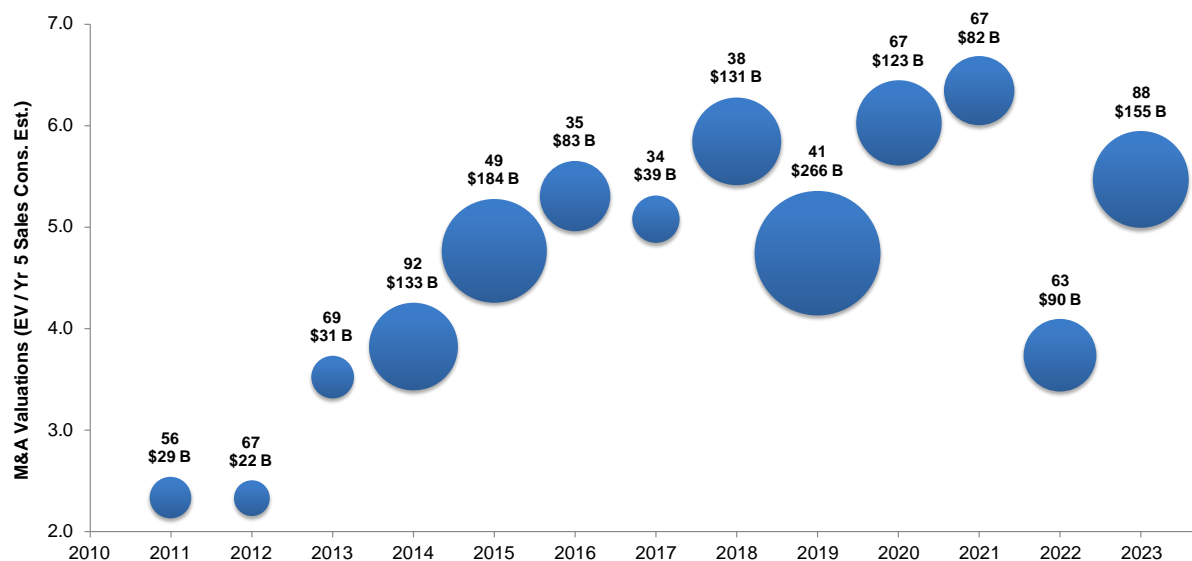
Source: Sachs Associates, Oppenheimer Research

Exhibit 3: We evaluated consensus estimates for a sample of drugs for rare neurological diseases and found they approach \$10B in annual revenues by the end of this decade providing ample incentive for investors and corporations who pursue research in these areas.



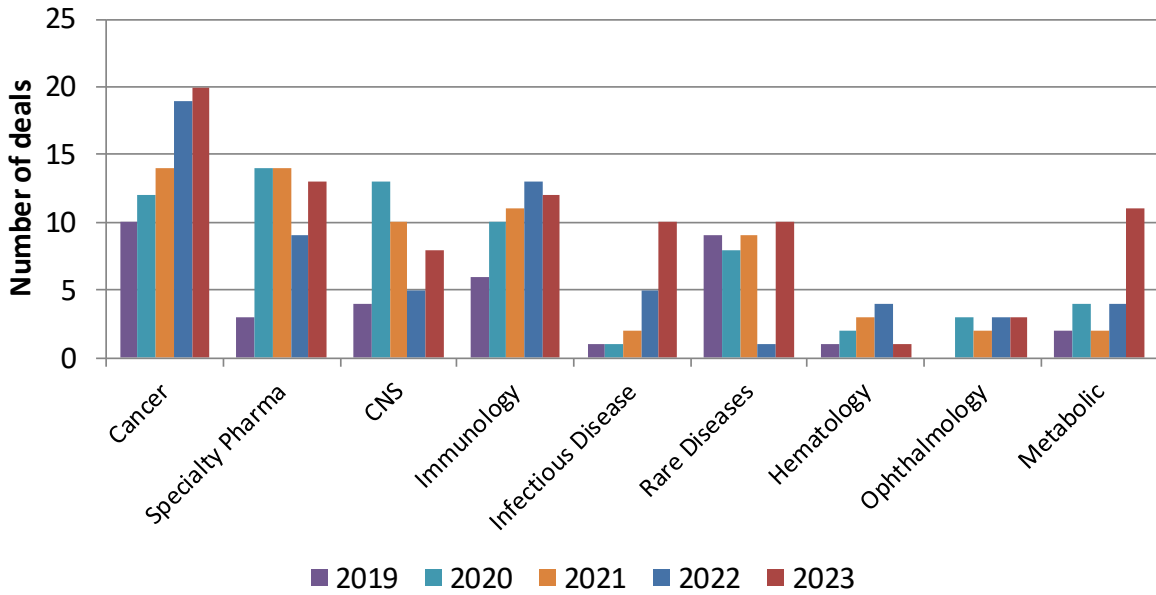
Source: Sachs Associates, Oppenheimer Research

Exhibit 4: Biotech/pharma M&A activity in 2023 was notably higher than 2022 based on both total number of deals at 88 vs. 63 and total transaction value of \$155B vs. \$90B, equating to a higher average transaction value of \$1.8B vs. \$1.4B. The average M&A valuation multiple of 5.5x EV/5-year cons. Est. sales in 2023 was higher than 3.7x in 2022, and consistent with the 5-year average of 5.3x, which could further expand as macro conditions improve. We view an overall multi-year trend of growing average deal sizes and higher multiples that we expect to continue in 2024.



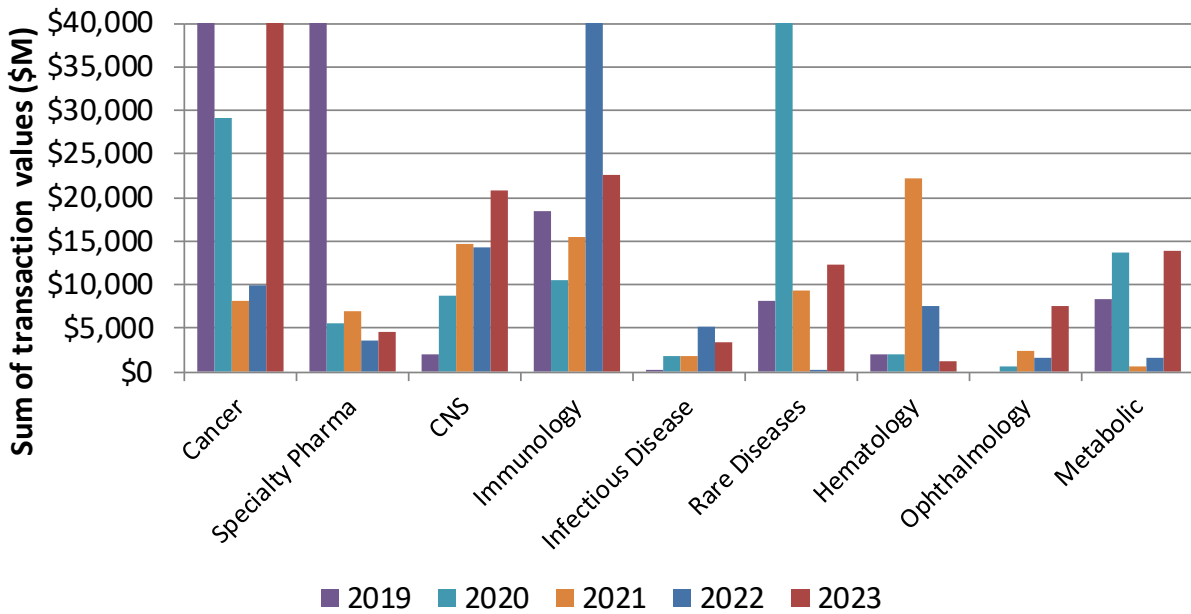
Source: FactSet, Oppenheimer & Co.

Exhibit 5: Biotech/pharma deal number in 2019-23 by therapeutic area shows cancer remains among the largest therapeutic targets of M&A ranked by deal number, with increasing numbers of metabolic, rare disease, and CNS deals in 2023 vs. prior years.



Source: Oppenheimer & Co.

Exhibit 6: Biotech/pharma transaction value in 2019-23 by therapeutic area shows cancer was the largest M&A target area by transaction value in 2023. CNS continues to increase over time, and we expect continued strategic interest in CNS assets in 2024.



Source: Oppenheimer & Co.

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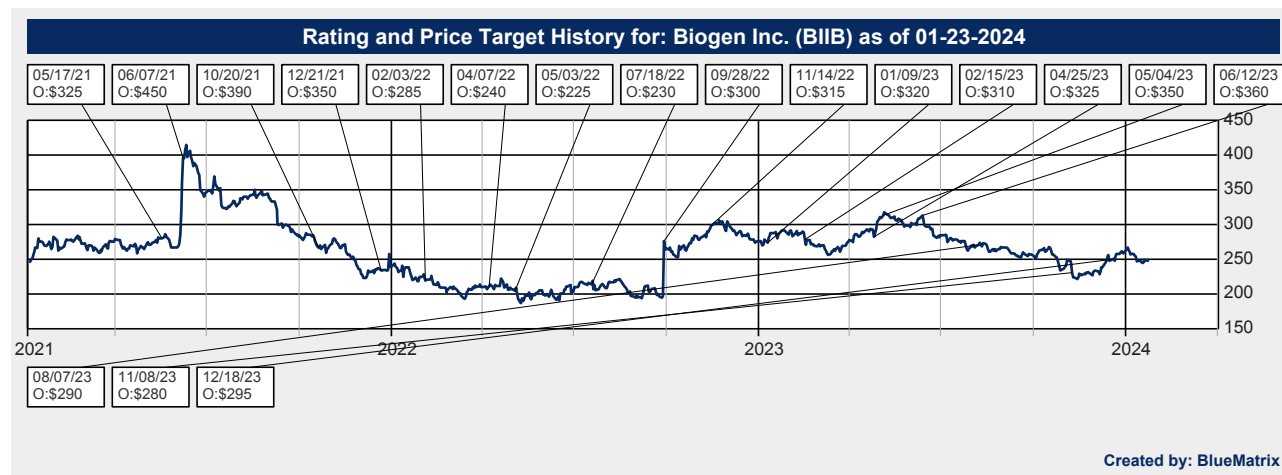
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Biogen Inc. (BIIB - NASDAQ, \$251.68, OUTPERFORM)



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Rating	IB Serv/Past 12 Mos.			
	Count	Percent	Count	Percent
OUTPERFORM [O]	431	62.37	196	45.48
PERFORM [P]	259	37.48	101	39.00
UNDERPERFORM [U]	1	0.14	0	0.00

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