

# Nxera Pharma

Leading the next era of medicine.  
From Japan, for Japan, and the world.

44th Annual J.P. Morgan Healthcare Conference

13 January 2026



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# Chris Cargill

President & CEO  
Nxera Pharma  
(TSE:4565)





Not a traditional Japanese pharma. We think and innovate globally, and specialize locally

## Drug Discovery Platform



CEO      Research      Finance      Chief of Staff      Legal

### Research & Early Clinical

- Cryo-EM Nobel Prize winning founder
- Proprietary StaR™ and NxWave™ structure-based drug design platform
- Complemented by AI-driven advances

### Technical Operations

- Global CMC Operations
- Supply Chain and Quality Management

**~200 team members**



## Commercial



Finance      Operation      Compliance



### Development & Commercial

- Bilingual management with global experience
- Agile, technology-enabled teams
- Novel go-to-market approaches

**~200 team members**



Our team is committed to addressing some of the biggest healthcare challenges globally



# Both sides of the business continue to advance rapidly

## Drug Discovery Platform



### Clinical Asset

#### Active Programs      Changes in 2025

Phase 3

2

+2

Phase 2

5

+2

Phase 1

8

-2 (moved to Ph2)

### Major Partners



abbvie

Lilly



Pfizer

## Commercial



### Products

#### 2025 Sales      Changes in 2025 (Median target: JPY)      (Median target)

PIVLAZ®

13.5Bn

+7%

QUVIVIQ®

4.5 Bn

+220%

### Late-stage assets

Vamorolone

New

Candidate drug for DMD. Already approved in US and Europe.

Lucerastat

Candidate drug for Fabry disease. Nxera have exclusive opt-in rights.

On track to continue growth trajectory throughout 2026

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Commercial business in Japan and APAC



# Our late-stage and commercialized assets in Japan and APAC



IN-HOUSE

Discovery – Preclinical

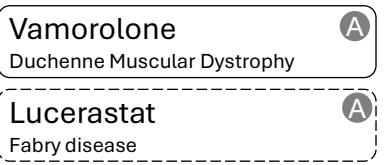
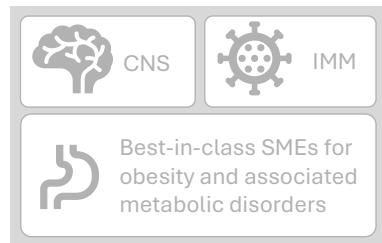
Phase 1

Phase 2

Phase 3

Filed

Commercial



**Commercial**

PARTNERED

Key discovery collabs.



Diabetes/Metabolic



Neurology

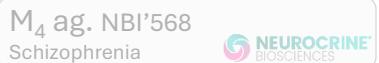
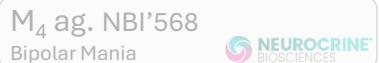
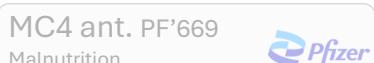
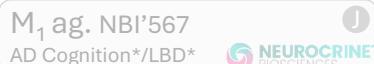
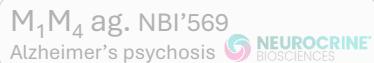
Key technology collabs.



AI-driven



Neurology & Autoimmune



[ ] : Exclusive license-in option

(A) : Nxera has APAC\* rights

(J) : Nxera has Japan rights

\*AD: Alzheimer's disease, LBD: Lewy Body Dementia

\*\*NXE0039732 (EP4 antagonist) is categorized as an in-house asset as we have not licensed out. Under the Clinical Trial and Licence Agreement (CTLA) in 2022, Cancer Research UK sponsors, designs and executes a Phase I/IIa clinical trial of NXE0039732, and Nxera holds a licence to the results generated under the trial to continue the clinical development and commercialization of NXE0039732.

\*\*As of late October 2025, Tempero Bio has temporarily halted advancement of the TMP-301 program and is assessing strategic alternatives

\*\*\*APAC (ex-China) territory includes Japan, South Korea, Australia, Brunei, Cambodia, Indonesia, Laos, Malaysia, Myanmar, New Zealand, Philippines, Singapore, Taiwan, Thailand and Vietnam

# Our two commercial products in Japan are showing significant sales growth yoy

Target 13.0 - 14.0 Bn JPY (PIVLAZ<sup>®</sup>) from net sales, and 4.0 - 5.0 Bn JPY (QUVIVIQ<sup>®</sup>) from royalties and supply

Target sales  
in FY2025

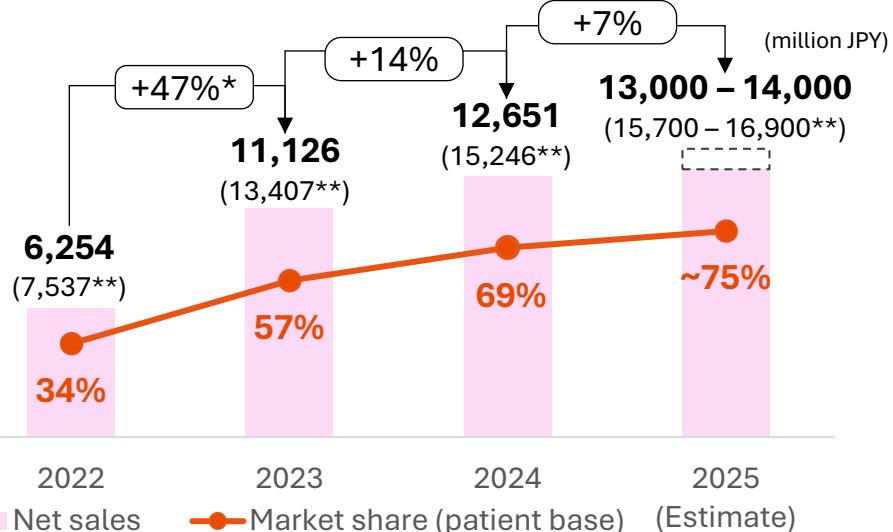


Sales trend



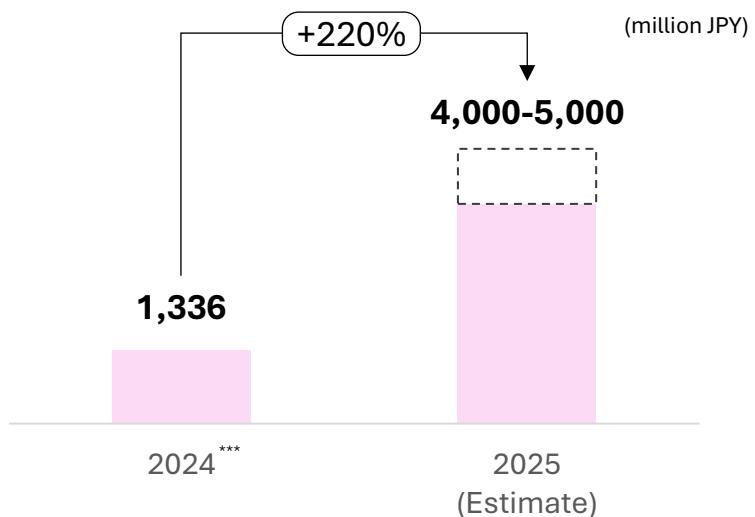
**13.0 – 14.0 Bn JPY**  
(NHI Sales: 15.7 – 16.9 Bn JPY)

+7%



**4.0 – 5.0 Bn JPY**  
(Shionogi: FY26/3E = 2.5 Bn JPY)

+220%



Source: MDV DPC hospital data

\*: Comparison of 2-4Q of 2022 and 2023, \*\* NHI sales, \*\*\* 2024 sales includes upfront, milestone, royalty and product supply while 2025 sales includes royalty and product supply

# Announced: In-licensing of vamorolone (AGAMREE®) for DMD

There is no established therapy for DMD other than corticosteroids in Japan

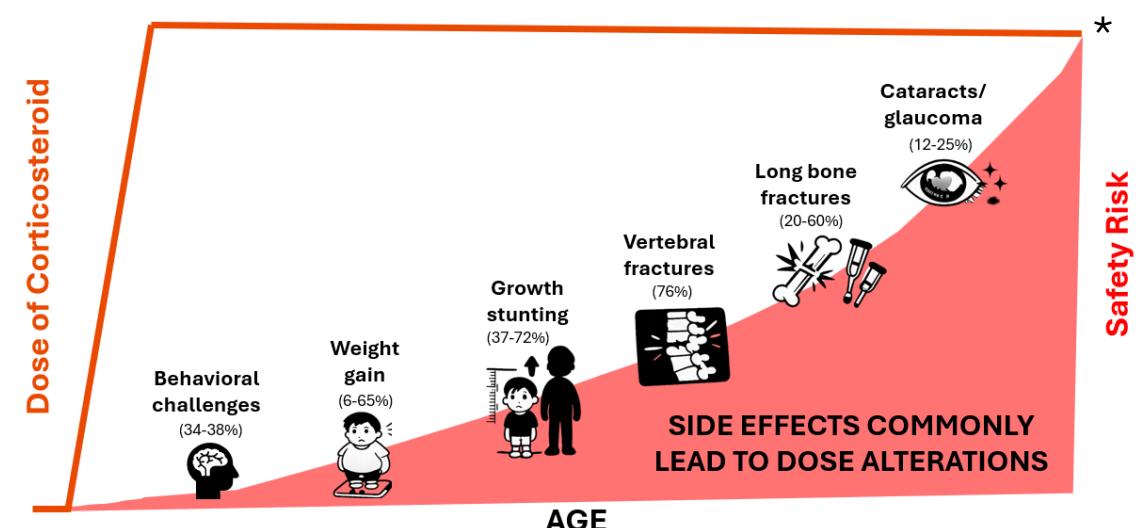
## Vamorolone (AGAMREE®)

- First-in-class drug candidate that binds to the **same receptors as corticosteroids** but modifies the downstream activity of the receptors
- Nxera has the development rights for **Japan, South Korea, Australia and New Zealand**
- DMD treatment is concentrated in a limited number of centers and there is approximately **70% sales synergy with PIVLAZ®**



## Duchenne Muscular Dystrophy (DMD)

- DMD is a rare and life-threatening neuromuscular disorder
- Characterized by progressive muscle dysfunction leading to ambulation loss, respiratory failure, heart issues and premature death
- No efficacious therapy apart from corticosteroids, however they present many severe adverse events





# Vamorolone (AGAMREE®) addresses the need for a tolerable steroid

Compared with conventional corticosteroid therapy, the risk of treatment-related adverse events is reduced

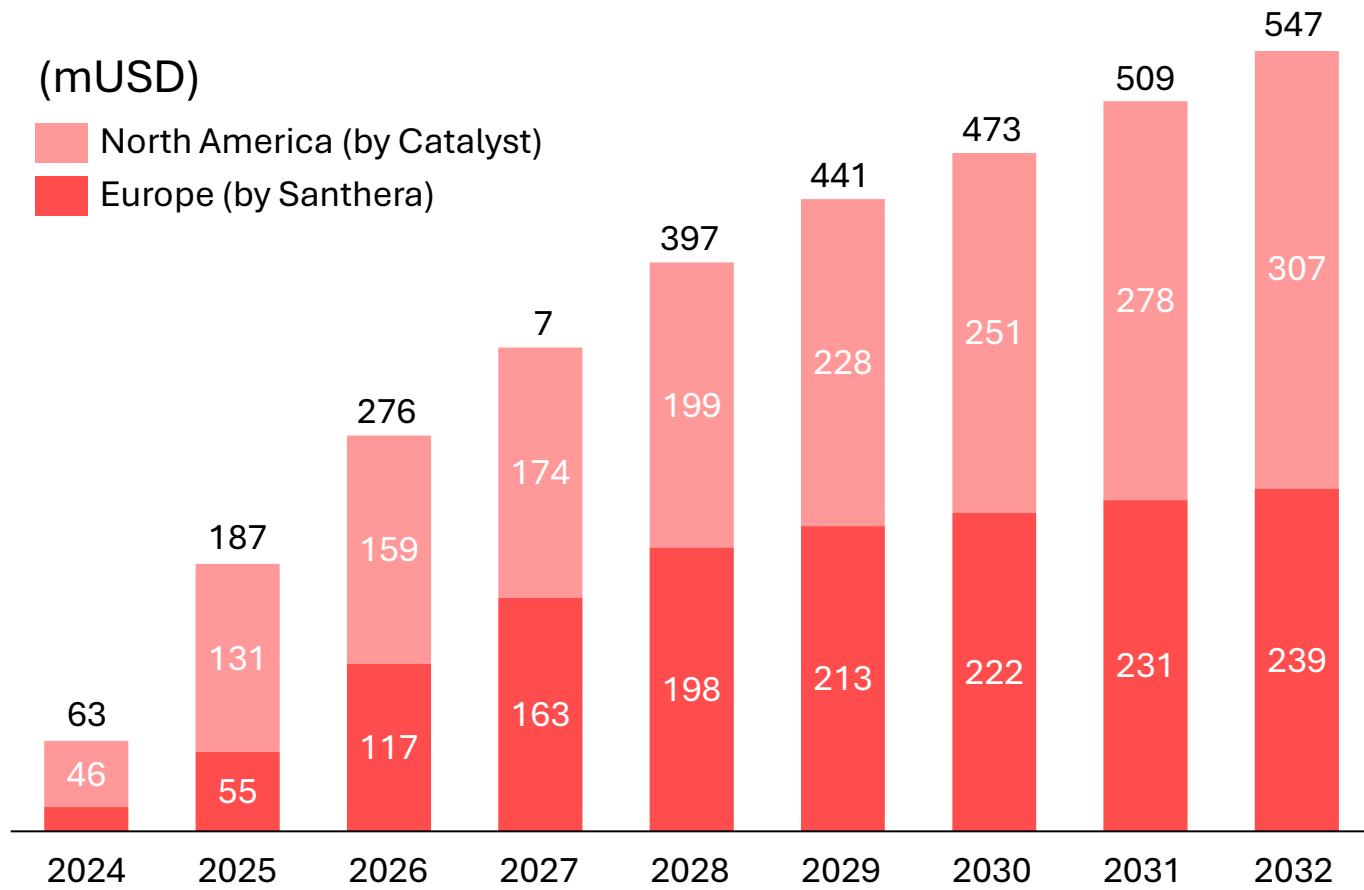


- Vamorolone confronts the limitations of standard corticosteroid therapy
- Topline data from the recent GUARDIAN clinical study showed **durable efficacy** and **markedly improved safety** of vamorolone vs. standard corticosteroids
- Study demonstrated reduction of steroid-associated adverse events related to:
  - Growth – *normal growth maintained (p<0.0001)*
  - Bone health – *lower vertebral fracture rate (p=0.0061)*
  - Eye health – *lower incidence of cataracts (p<0.015) and no cases of glaucoma*
- Reduction of side effects allows patients **to maintain treatment**

## Consensus sales forecast of vamorolone in other countries

(mUSD)

North America (by Catalyst)  
Europe (by Santhera)

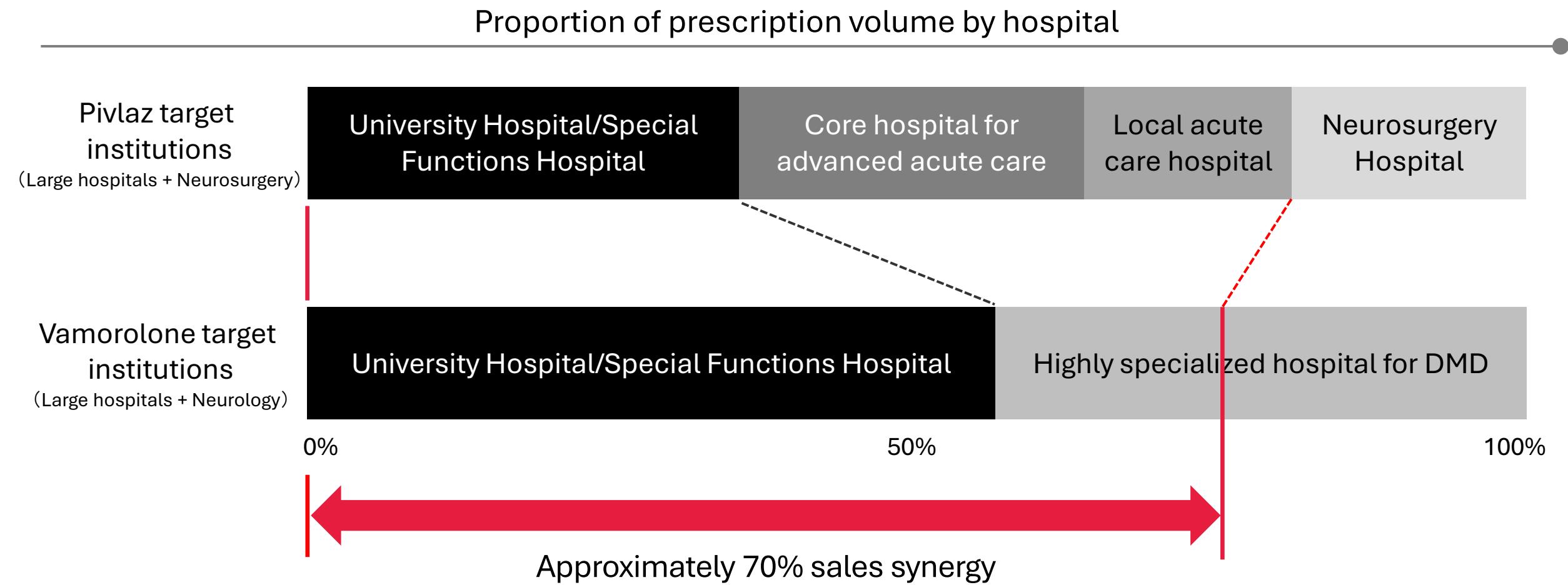




## Synergy with Pivlaz



DMD treatment is concentrated in a limited number of centers and there is approximately 70% commercial overlap with PIVLAZ, creating significant sales synergies



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# Drug Discovery Platform & Pipeline Update

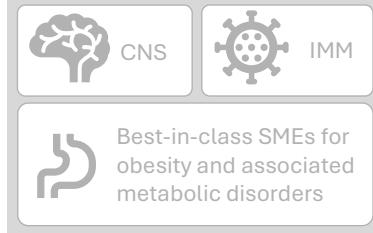




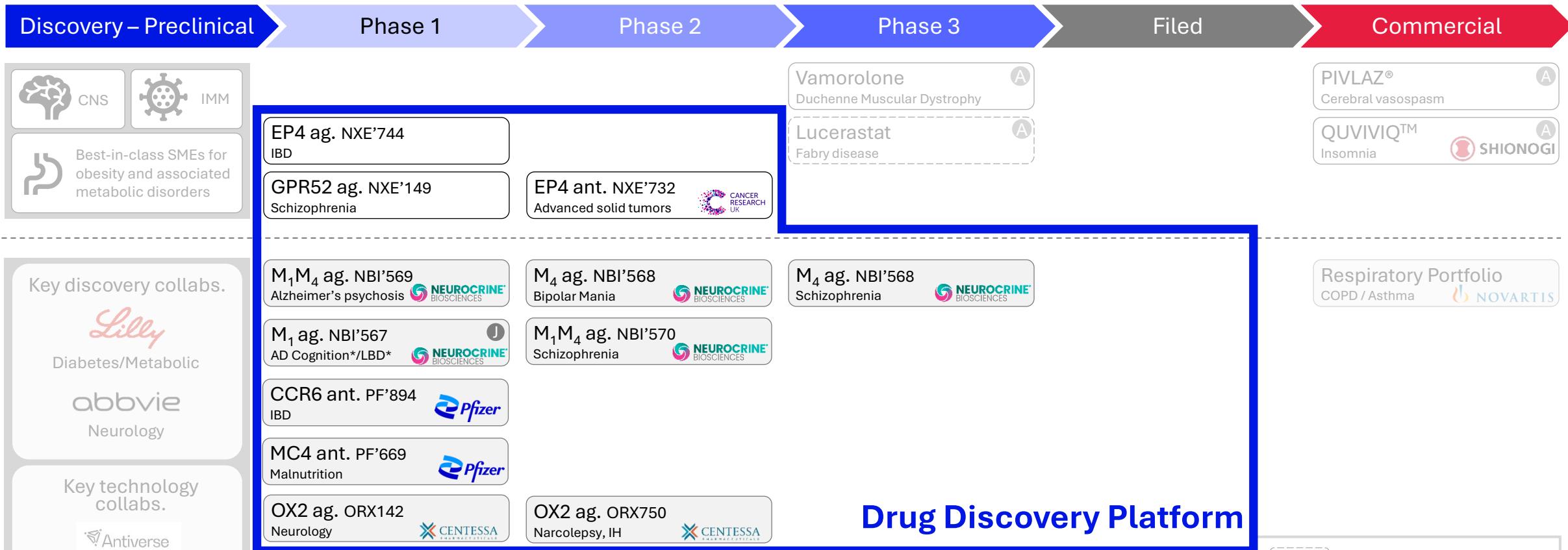
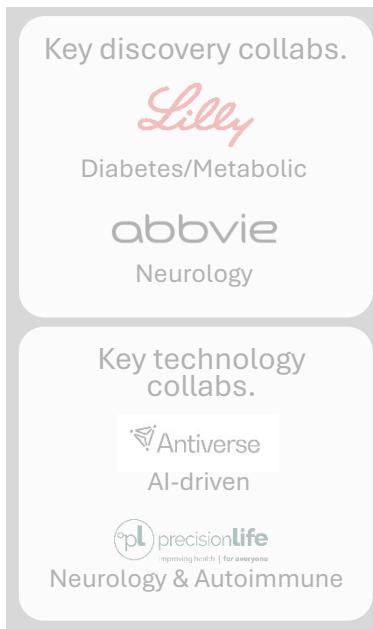
# Portfolio of drugs developed using our innovative NxWave™ platform



IN-HOUSE



PARTNERED



## Drug Discovery Platform

[ ] : Exclusive license-in option  
 A : Nxera has APAC\* rights  
 J : Nxera has Japan rights

\*AD: Alzheimer's disease, LBD: Lewy Body Dementia

\*\*Nxera (ex-China) territory includes Japan, South Korea, Australia, Brunei, Cambodia, Indonesia, Laos, Malaysia, Myanmar, New Zealand, Philippines, Singapore, Taiwan, Thailand and Vietnam.

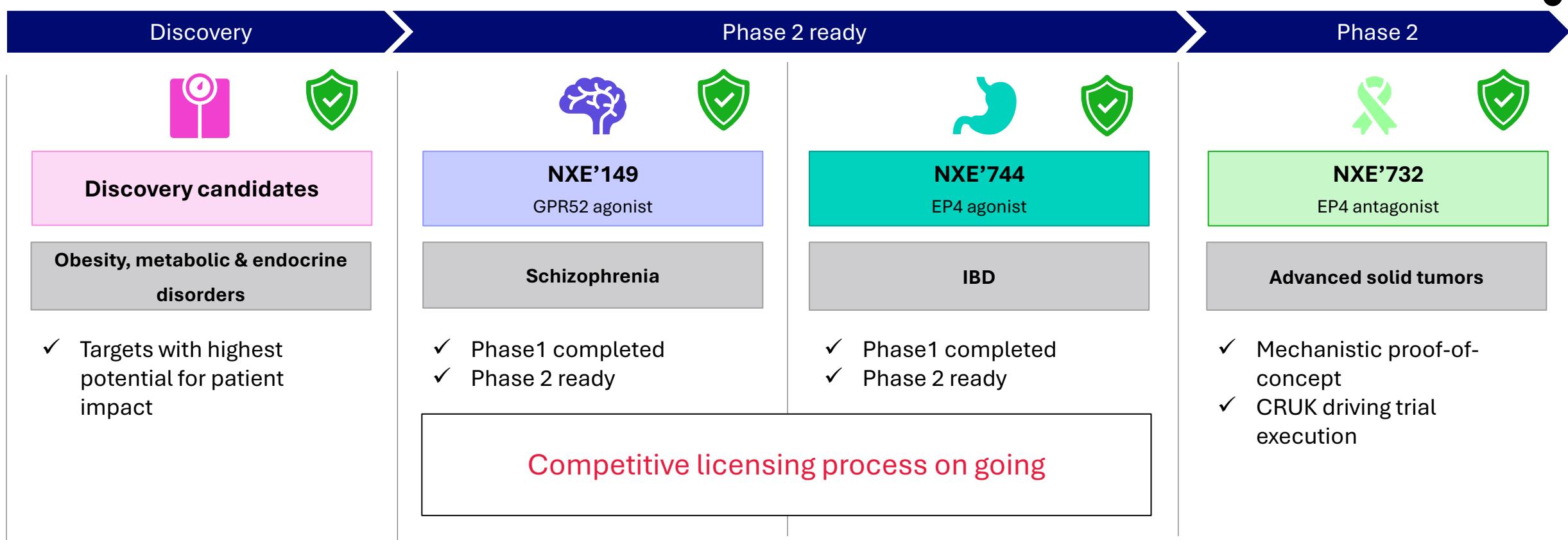
\*\*APAC (ex-China) territory includes Japan, South Korea, Australia, Brunei, Cambodia, Indonesia, Laos, Malaysia, Myanmar, New Zealand, Philippines, Singapore, Taiwan, Thailand and Vietnam.



# Renewed R&D focus where the science is strongest and the opportunity is greatest



## IN-HOUSE PORTFOLIO - R&D FOCUS AND PROGRAM PRIORITISATION



R&D focus on highest potential opportunities



# NXE'149: GPR52 agonist for schizophrenia – Phase 2 ready

A novel first-in-class mechanism to treat positive, negative & cognitive domains of schizophrenia



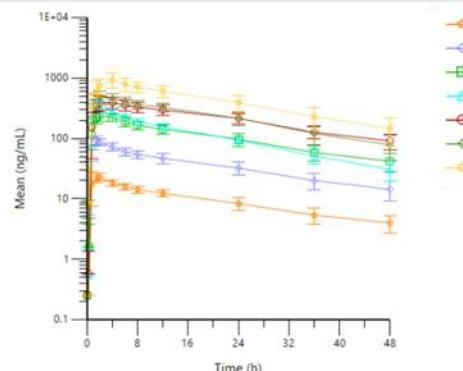
## Phase 1 highlights:

- ✓ Safe and well tolerated
- ✓ Human PK showed low variability and consistent with once daily dosing
- ✓ High level of central penetration
- ✓ Pharmacodynamic measures provide evidence of engagement of brain circuitry relevant to the treatment of schizophrenia and related disorders

## Phase 2 enablement:

- 3 month GLP toxicology in 2 species
- 2 species EFD completed
- Metabolite characterisation complete
- Drug substance and drug product available for phase 2 start

### SAD PK data



### EEG and ERP measures

- NXE'149 clearly engages frontotemporal circuitry underlying the MMN and ASSR responses, both of which are reproducible biomarkers in schizophrenia
- Resting state EEG data suggest increased arousal on day 10 of treatment

### Cognition

Cogstate assessment demonstrated improvements in cognitive performance across doses on day 10 of treatment

General cognitive composite	Dose 1	Dose 2	Dose 3	Dose 4
Attention/Executive Function	0.89	1.5	0.69	0.64
General Cognition	1.1	0.84	0.77	0.55

Standardized differences between each dose of NXE'149 compared to placebo



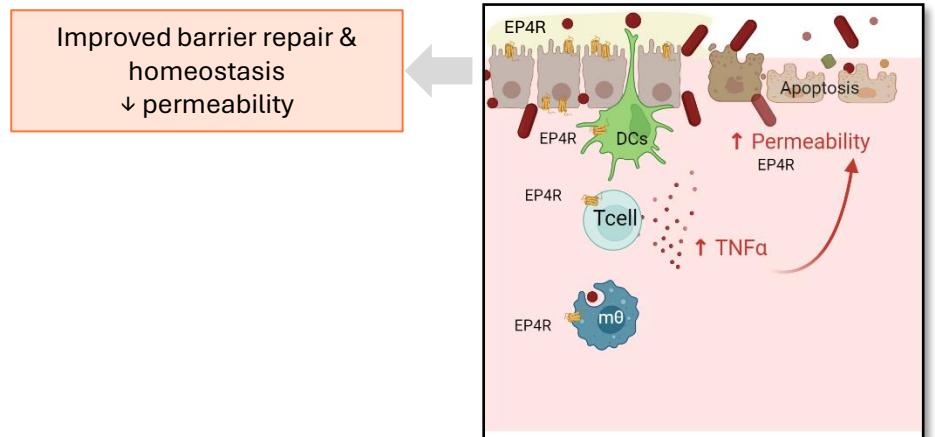
# NXE'744: EP4 agonist for inflammatory bowel disease (IBD) – Phase 2 ready

A first-in-class GI-targeted agent to promote mucosal healing in IBD



## Disease Rationale

- All approved IBD agents are immunomodulatory in nature and do not directly target disease-induced mucosal barrier defects
- Through combined anti-inflammatory and barrier repair effects, EP4 agonists are expected to bring benefits in IBD by promoting mucosal healing
- Previous attempts to agonise the EP4 receptor have demonstrated early signals of clinical efficacy but have been limited by systemic safety



## Progress

- **All elements of the first-in-human study have now completed dosing in the clinic**
- **SAD/MAD studies are complete** with no concerning adverse events noted to date and no systemic exposure observed
- **Gut restricted profile confirmed** by high gut tissue concentrations measured following oral dosing
- **UC patient cohort has completed dosing (n=6)** with data read-out (PK measurements) imminent.
- **Indomethacin challenge cohort 1 complete** with final data read-out by March 2026 (interim analysis ongoing)
- Biomarker data analysis from Ph1 studies in progress to inform project strategy

Study link:

<https://www.isrctn.com/ISRCTN70080074?q=nxera&filters=&sort=&offset=1&totalResults=2&page=1&pageSize=10>

## NXE'732: EP4 antagonist is our novel immunotherapy for solid tumors

## Phase 2a expansion in process in combination with atezolizumab

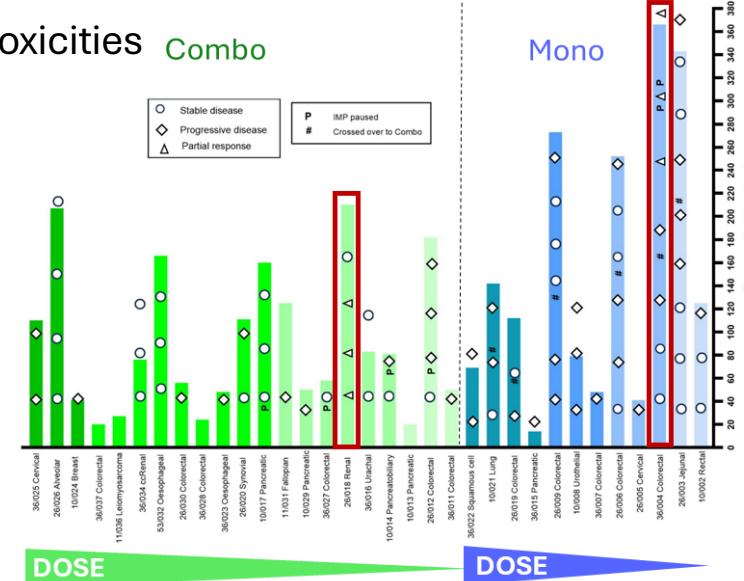
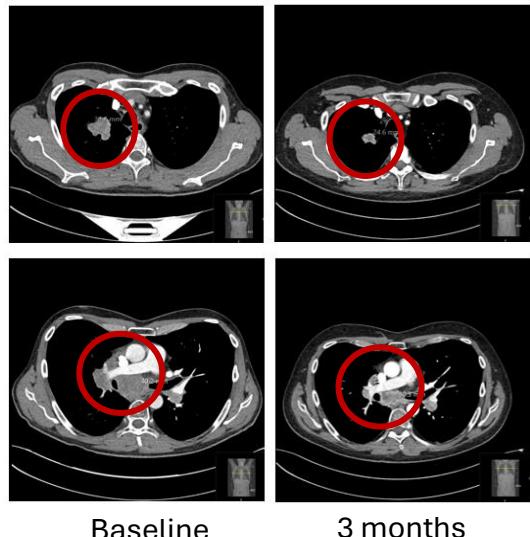


## Disease Rationale

- When EP4 is activated, it dampens immune responses and promotes tumor growth
- EP4 antagonism is a highly attractive mechanism supported by recent clinical data for ONO-4578 in gastric cancer
- NXE-732 is designed to deliver **high potency, selectivity, and safety**

## Phase 1 trial results

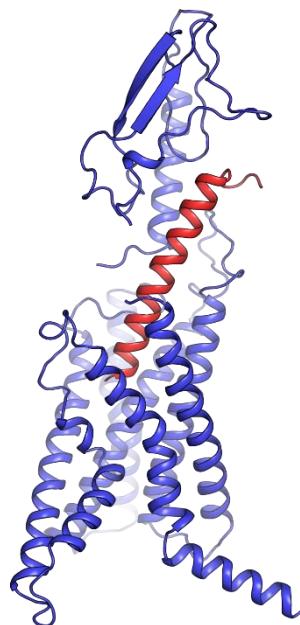
- The emerging data for NXE-732 points to a potential best-in-class profile
- Two partial responses were observed in MSS CRC and anti-PD-L1 resistant ccRcc in the combination arm, with meaningful tumor shrinkage of over 30% demonstrated
- Target engagement confirmed and no dose-limiting toxicities



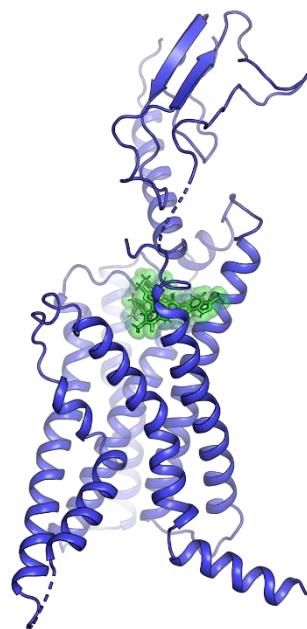
## Phase 2a expansion study underway in **MSS Colorectal** (PIK3CA, HER2± others), **Gastric/GOJ Adenocarcinoma, Renal (ccRCC), Prostate (CRPC)**

We can make a huge impact by leveraging our GPCR expertise in the areas of highest unmet medical need: next-generation small molecules for obesity, metabolic and endocrine disorders

## Unparalleled GPCR SBDD capabilities



Structure of GLP1-R  
bound to **peptide**



Structure of GLP1-R  
bound to **small molecule**

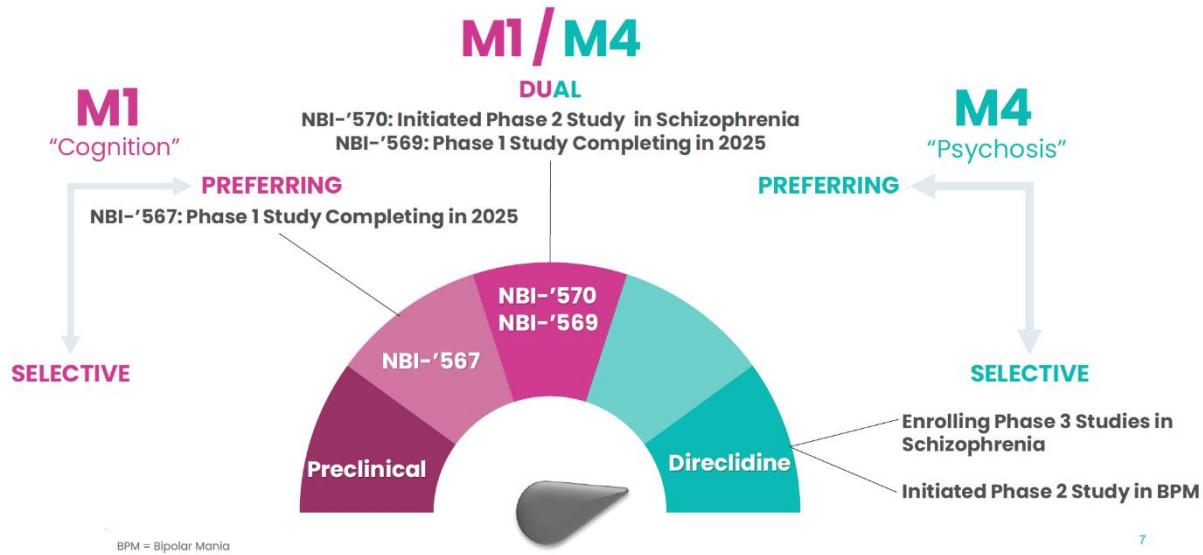
- **Launched broad new pipeline**, advancing next-gen BIC therapies for obesity and metabolic disorders
- **Convenient, scalable oral therapies** for sustained weight loss in a market dominated by peptides
- **Targeting key obesity-related co-morbidities**: Enhanced outcomes in cardiovascular, renal, and liver diseases
- **Reducing side effects** and **broadening** out to difficult to treat populations

MECHANISM	Nxera
GLP-1 ag	
GIP ant	
Amylin ag	
Multiple other targets of interest	

Nxera aims to redefine obesity, weight management and related co-morbidities by delivering potent, oral small molecules to meet a critical global need at scale



Neurocrine is advancing the world's most comprehensive portfolio of muscarinic agonists to treat neuropsychiatric disorders



Program	Mechanism	Disease State	Stage of Development
<b>Direclidine</b>	M4 Agonist	Schizophrenia	Phase 3
		Bipolar Mania	Phase 2
<b>NBI-570</b>	Dual M1/M4 Agonist	Schizophrenia / LAI Potential	Phase 2
<b>NBI-569</b>	Dual M1/M4 Agonist	Alzheimer's Psychosis	Entering Phase 1b
		Alzheimer's Cognition	Phase 1
<b>NBI-567</b>	M1 Preferring Agonist	Lewy Body Dementia	

Five clinical-stage programs spanning the M1, M4, and dual M1/M4 mechanisms designed using NxWave™



# Centessa is advancing ORX750, a potential best-in-class Orexin Receptor 2 agonist for treatment of NT1, NT2 and IH



## Potential BIC for NT1, NT2 and IH

ORX750

CRYSTAL-1 Phase 2a study in NT1, NT2 and IH



Evaluate safety, tolerability, and PK in NT1, NT2, and IH patients

Efficacy assessment registrational endpoints: **Maintenance of Wakefulness Test (MWT)**, **Epworth Sleepiness Scale (ESS)**, **weekly cataplexy rate** (NT1 patients only), and overall symptom improvement\*

Exploratory efficacy assessments will measure sleep, **cognition, attention, memory**, and general health

**First robust demonstration** of oral OX2R agonist addressing wakefulness needs of patients across NT1, NT2 and IH...

- ✓ **Generally favorable safety and tolerability profile**
- ✓ **Statistically significant, clinically meaningful and dose-dependent efficacy**
- ✓ **Dose escalation** across ongoing and future cohorts with **once-daily and split-dose regimens**, enabled by Phase 1 data

...Expect to initiate registration program in Q1 2026

## Phase 2a study update

### Endpoints

Maintenance of Wakefulness Test (MWT)	>20 min change at 1.5mg vs baseline (with half of participants >30 min). NT1 >10 min change at 4mg vs baseline. NT2
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Epworth Sleepiness Scale (ESS)	1.5mg = 5.1 vs 18.7 (placebo). NT1 4mg = 8.1 vs 15.9 (placebo). NT2
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Weekly Cataplexy Rate (WCR)	87% relative reduction at 1.5mg vs placebo. NT1
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Participants	55 participants (NT1, NT2 & IH)
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Next step	Registrational Program initiation planned for Q1 2026
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Initial Phase 2a data mark first robust demonstration of oral OX2R agonist addressing wakefulness needs of patients across all three indications; **Expect to initiate registrational program in Q1 2026**

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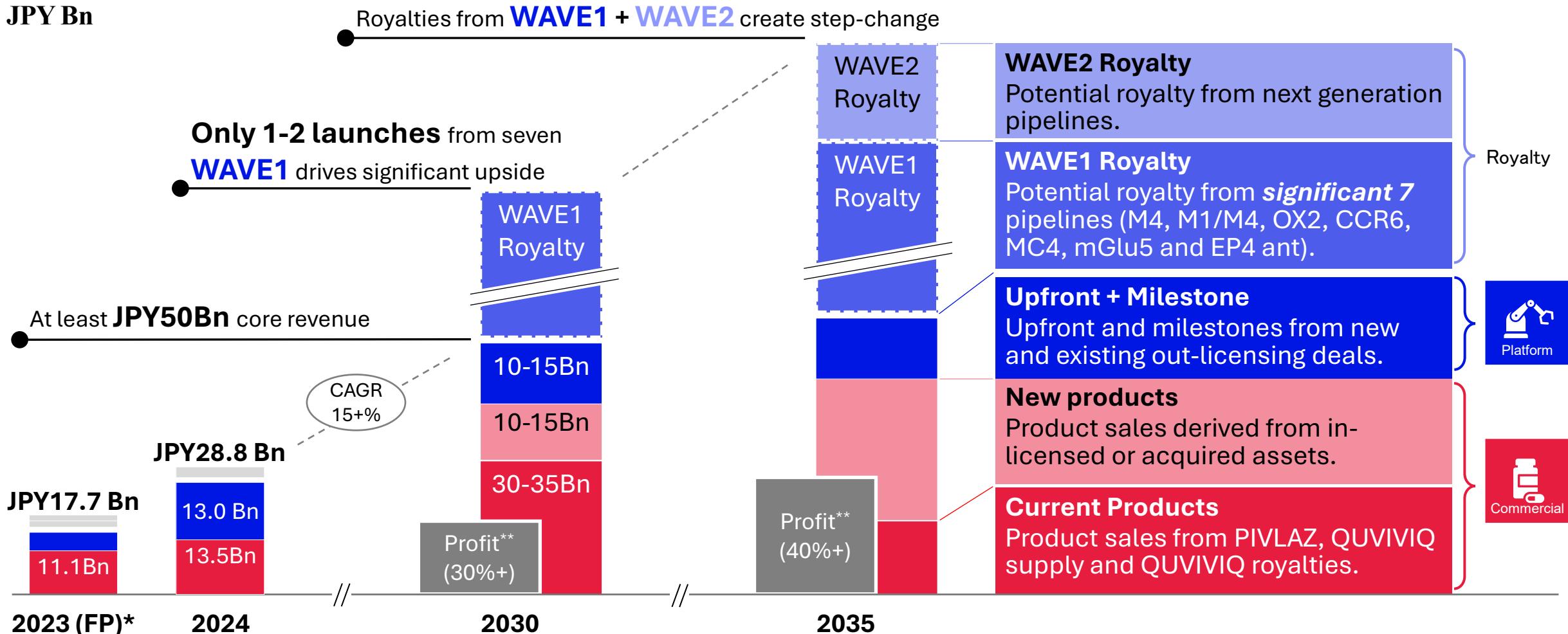
# The Big Picture





# Our 2030 vision is to build a high growth, highly profitable Japanese biopharma

JPY Bn



Note: \* Revenue values are proforma the acquisition of Idorsia Pharmaceuticals Japan and Korea and reflect annual product sales of PIVLAZ in 2023.

\*\* WAVE1 and WAVE2 royalty is not included.

\*\*\* As of late October 2025, Tempero Bio has temporarily halted advancement of the TMP-301 program and is assessing strategic alternatives



# Looking ahead to potential catalysts in 2026\*

PROGRAM	PARTNER	TIMING	EVENT
ORX750 (OX2 agonist)	 CENTESSA PHARMACEUTICALS	Q1 2026	Phase 2a data across NT1, NT2, and IH
ORX750 (OX2 agonist)	 CENTESSA PHARMACEUTICALS	Q1 2026	Registrational program start in NT1/NT2/IH
ORX142 (OX2 agonist)	 CENTESSA PHARMACEUTICALS	Q1 2026	Phase 2 study start
ORX489 (OX2 agonist)	 CENTESSA PHARMACEUTICALS	Q1 2026	Phase 1 study start
NBI'570 (M1/M4 ago)	 NEUROCRINE BIOSCIENCES	Q1 2026	Phase 2 study start
Multiple discovery collaboration progress	abbvie 	1H 2026	Progression through discovery stage
Cenerimod	 VIATRIS™	Q4 2026	Phase 3 data readout
Muscarinic agonist	 NEUROCRINE BIOSCIENCES	2H 2026	Clinical progression
PF'894 (CCR6 antagonist)	 Pfizer	2026	Phase 1 data readout
PF'669 (MC4 antagonist)	 Pfizer	2026	Phase 1 data readout
NBI'567 (M1 ago) / NBI'569 (M4 ago) / NBI'570 (M1/M4 ago)	 NEUROCRINE BIOSCIENCES	2026	Phase 1 data disclosure
New global out-licenses		Anytime	Out licensing and/or discovery collabs
New Japan / APAC in-licenses		Anytime	Acquire/in-license late-stage medicines
QUVIVIQ™		Anytime	APAC out-licensing deals

Rapidly executing on our 2030 vision to be Japan's high growth, emerging biopharma champion

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# Questions?

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Thank you

