



**Bring**  
The full potential of  
our innovative  
medicines to patients



**Build**  
A high-value  
sustainable pipeline



**Deliver**  
Efficiencies to  
enable targeted  
investment &  
growth



**Boost**  
A culture of  
collaboration  
& excellence

# 41st Annual J.P. Morgan Healthcare Conference

January 2023



*Focus. Together.  
For patients & society*

# Disclaimer & safe harbor

- This presentation includes only summary information and does not purport to be comprehensive. Forward-looking statements, targets and estimates contained herein are for illustrative purposes only and are based on management's current views and assumptions. Such statements involve known and unknown risks and uncertainties that may cause actual results, performance or events to differ materially from those anticipated in the summary information. Actual results may depart significantly from these targets given the occurrence of certain risks and uncertainties, notably given that a new medicine can appear to be promising at a preparatory stage of development or after clinical trials but never be launched on the market or be launched on the market but fail to sell notably for regulatory or competitive reasons. Ipsen must deal with or may have to deal with competition from generic medicines that may result in market-share losses, which could affect its level of growth in sales or profitability. The Company expressly disclaims any obligation or undertaking to update or revise any forward-looking statements, targets or estimates contained in this presentation to reflect any change in events, conditions, assumptions or circumstances on which any such statements are based, unless so required by applicable law.
- All medicine names listed in this document are either licensed to Ipsen or are registered trademarks of Ipsen or its partners.
- The implementation of the strategy has to be submitted to the relevant staff representation authorities in each country concerned, in compliance with the specific procedures, terms and conditions set forth by each national legislation.
- In those countries in which public or private-health cover is provided, Ipsen is dependent on prices set for medicines, pricing and reimbursement-regime reforms and is vulnerable to the potential withdrawal of certain medicines from the list of reimbursable medicines by governments, and the relevant regulatory authorities in its locations. In light of recent economic conditions, there could be increased pressure on the pharmaceutical industry to lower medicine prices.
- Ipsen operates in certain geographical regions whose governmental finances, local currencies or inflation rates could erode the local competitiveness of Ipsen's medicines relative to competitors operating in local currency, and/or could be detrimental to Ipsen's margins in those regions where Ipsen's sales are billed in local currencies.
- In a number of countries, Ipsen markets its medicines via distributors or agents; some of these partners' financial strengths could be impacted by changing economic or market conditions, potentially subjecting Ipsen to difficulties in recovering its receivables. Furthermore, in certain countries whose financial equilibrium is threatened by changing economic or market conditions, and where Ipsen sells its medicines directly to hospitals, Ipsen could be forced to lengthen its payment terms or could experience difficulties in recovering its receivables in full.
- Ipsen also faces various risks and uncertainties inherent to its activities identified under the caption 'Risk Factors' in the Company's [Universal Registration Document](#).
- All of the above risks could affect Ipsen's future ability to achieve its financial targets, which were set assuming reasonable macroeconomic conditions based on the information available at the time.

# A future focused on Specialty Care

*Consumer HealthCare divested last year*

## Our vision

To be a leading global, mid-sized biopharmaceutical company with a focus on transformative medicines in Oncology, Rare Disease & Neuroscience



### ONCOLOGY

Strengthening  
the position



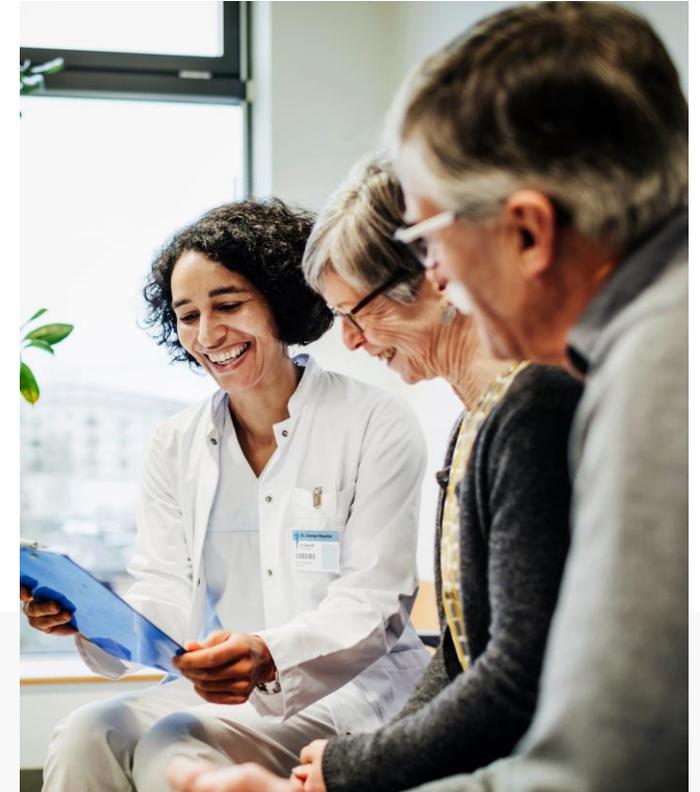
### RARE DISEASE

Expanding  
the scope



### NEUROSCIENCE

Excelling  
& accelerating



# Albireo: expanding Ipsen's scope in Rare Disease

*Perfectly aligned to the external-innovation strategy*

## Global rights<sup>1</sup>

- Bylvay: a potentially best-in-class rare liver-disease medicine approved in the U.S. & E.U.

## Strategic fit

- Expanding the pipeline & portfolio in rare liver diseases

**Albireo** 

## Multiple opportunities

- Bylvay: progressive familial intrahepatic cholestasis, Alagille syndrome, biliary atresia
- Earlier-stage pipeline: adult cholestatic liver diseases

## Financial impact

- Sizeable peak sales ~\$800m
- Accretive to core operating income from 2025

# The Ipsen investment case

Entire focus  
on Specialty  
Care



Opportunities for  
further growth  
across the three  
therapy areas



Global  
footprint

A well-balanced  
& expanded  
presence  
around the world

Expanding  
pipeline



A good mix of  
new molecules  
and lifecycle  
management



External-  
innovation  
strategy

16 assets  
in 18 months  
across the three  
therapy areas

Strong balance  
sheet & cash  
generation



>€2bn  
firepower<sup>1</sup>  
by 2024  
Free cash flow  
>€800m in 2021



**Maximize brands**



**Strengthen pipeline**



# The Ipsen strategy



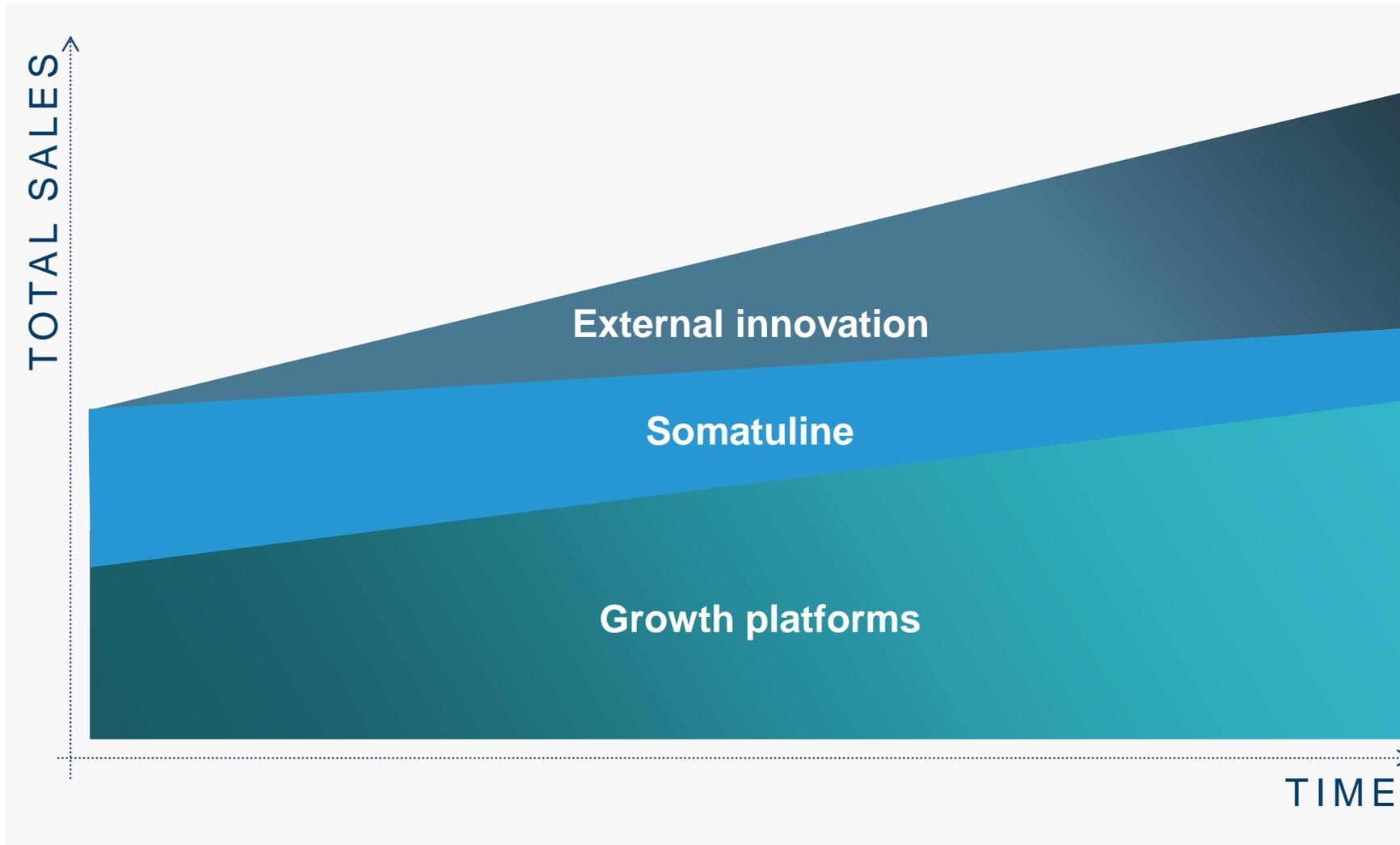
**Drive efficiencies**



**Focus on culture**

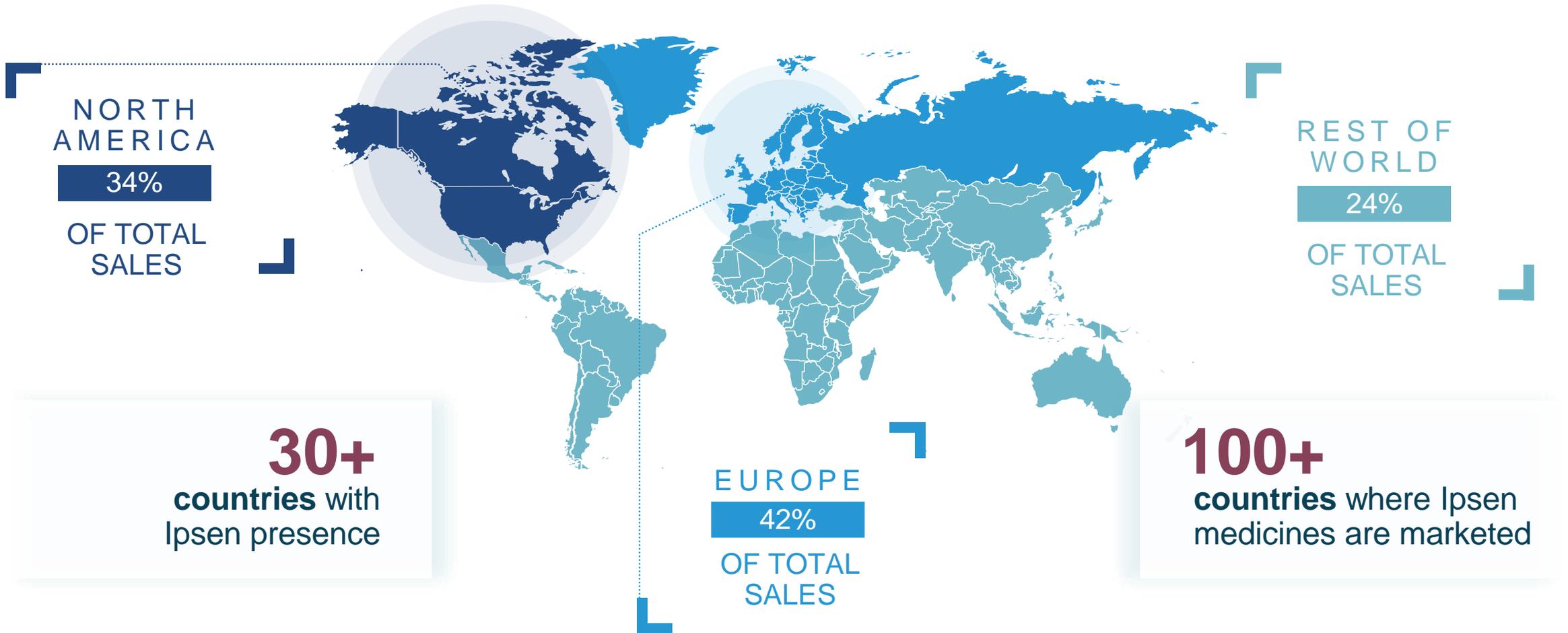


# A strong platform for sustainable growth



- Accelerate growth with external innovation
- Transition post-SSA competition entry
- Drive performance of growth platforms

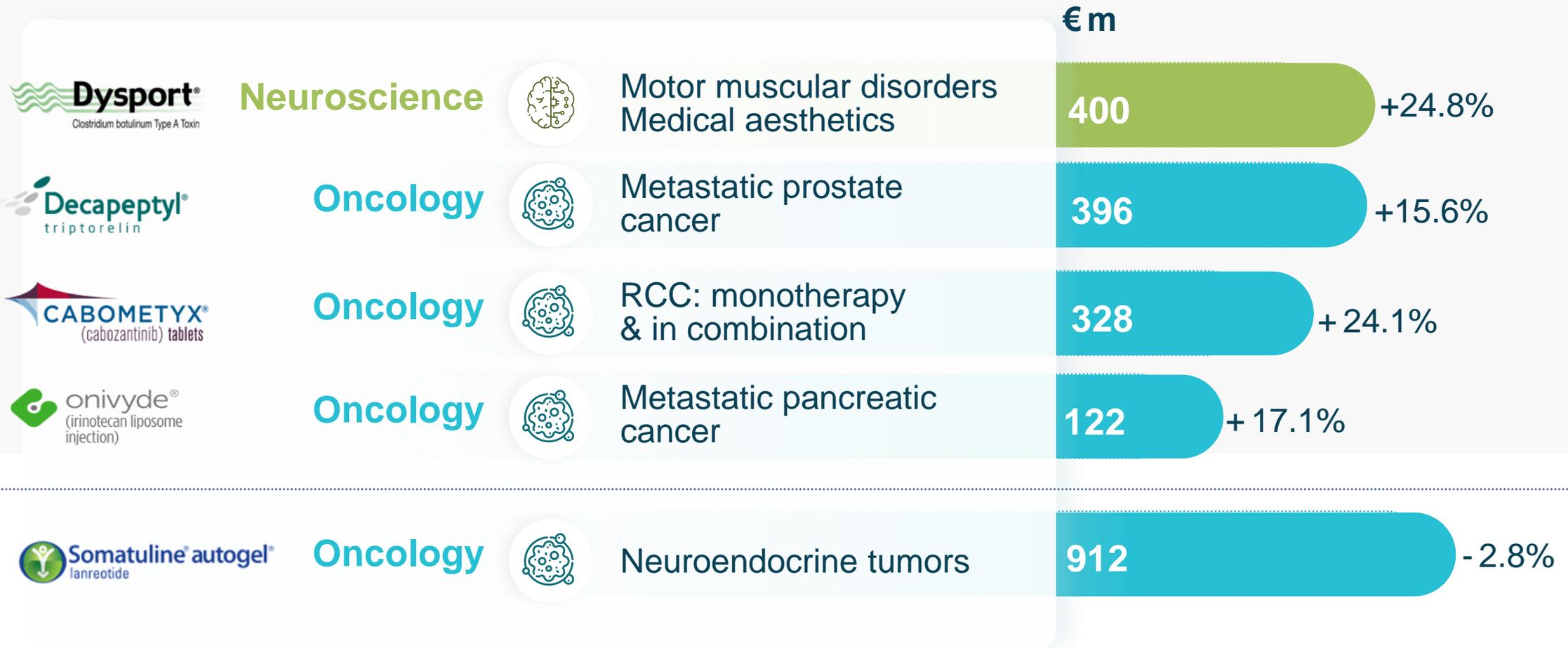
# A strong & expanded global footprint



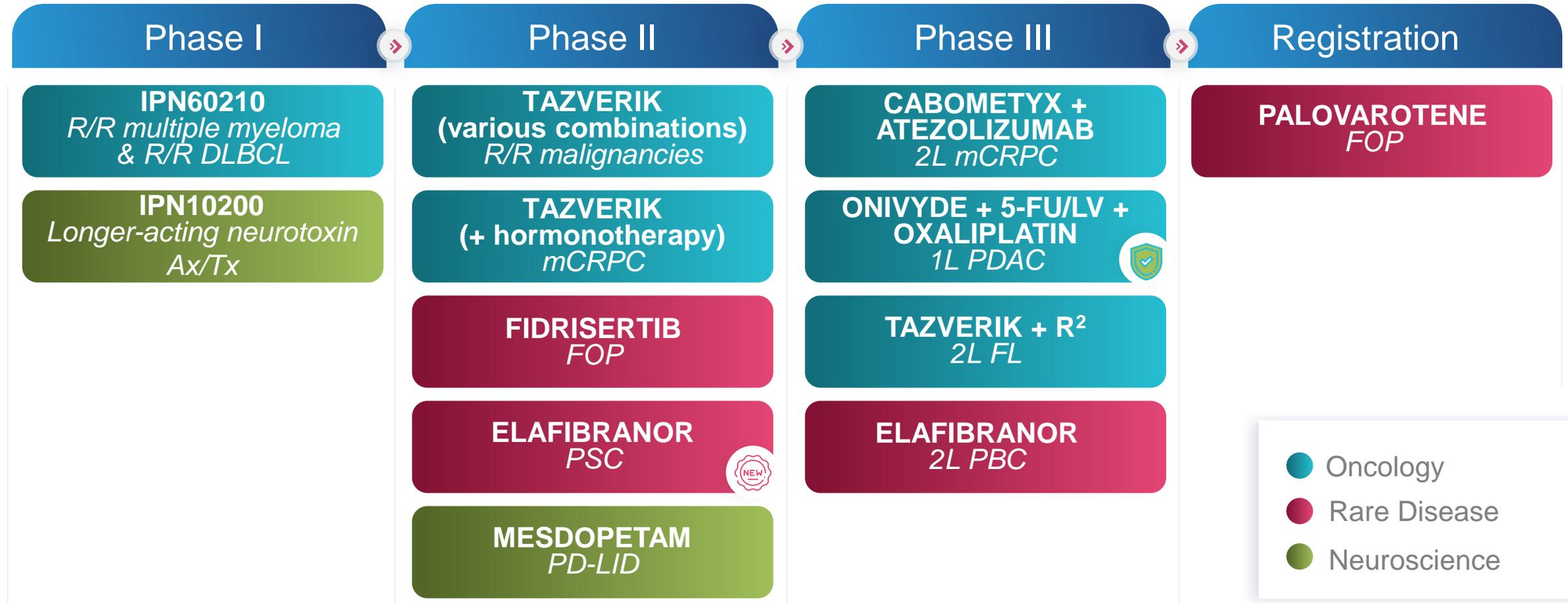
# September year to date 2022: sales increased by 9.5%

Growth platforms up by 20.8%

GROWTH  
PLATFORMS



# Building a high-value, sustainable pipeline



Information shown as at the end of December 2022. **R/R**: relapsed/refractory; **DLBCL**: diffuse large B-cell lymphoma; **Ax**: aesthetics; **Tx**: therapeutics; **mCRPC**: metastatic castration-resistant prostate cancer; **FOP**: fibrodysplasia ossificans progressiva; **PSC**: primary sclerosing cholangitis; **PD-LID**: Parkinson's disease - levodopa-induced dyskinesia; **2L**: second line; **1L**: first line; **PDAC**: pancreatic ductal adenocarcinoma; **R<sup>2</sup>**: lenalidomide + rituximab; **FL**: follicular lymphoma; **PBC**: primary biliary cholangitis.

# Pipeline: near-term major milestones



## Palovarotene: FOP

Regulatory decisions - U.S.<sup>1</sup>, E.U.



## Mesdopetam: PD-LID

Phase IIb data readout



## Elafibranor: 2L PBC

Phase III data readout



# Consistent execution of the external-innovation strategy

16 assets in the last 18 months



## ONCOLOGY: 12 assets

**Tazverik**  
Epizyme

Approved

**ERK-**  
**inhibitor**  
AGV Discovery

Preclinical

**METTL3**  
Accent  
Therapeutics

Preclinical

**BKX-001**  
BAKX  
Therapeutics

Preclinical

**FLIP-i**  
programme  
QUB

Preclinical

**IO**  
Marengo

Preclinical



## RARE DISEASE: 1 asset

**Elafibranor**  
GENFIT

Phase III



## NEUROSCIENCE: 3 assets

**Mesdopetam**  
IRLAB

Phase IIb

**SNAs**  
Exicure

Preclinical

**BoNT/X**  
BCH/UOS

Preclinical

# Elafibranor

Peak-sales outlook: around €500m

In Phase III clinical development for 2L PBC - data anticipated in H1 2023

**Expanding Ipsen's position in Rare Disease**

High unmet medical need

**A first-in-class, innovative potential treatment option**

U.S. prevalence: 23.9-39.2 per 100,000<sup>1, 2</sup>

**Compelling Phase II data**

Breakthrough Therapy & Orphan Drug Designations

**Exclusive worldwide licence<sup>3</sup>**

to develop, manufacture & commercialize elafibranor

**Beyond PBC: ELMWOOD Phase II trial initiated in PSC**

# Tazverik

**TAZVERIK**  
(tazemetostat) tablets  
200 mg

*Longer-term peak-sales outlook: up to \$800m*

## In Phase III clinical development for 2L+ FL - data anticipated in 2026

### Building on Ipsen's commitment to Oncology

Replenishing the pipeline & leveraging the U.S. presence

### First-in-class EZH2 inhibitor approved in 3L FL

U.S. on-market compound with good patent life

### Strength of data supporting Tazverik's positioning

An oral, efficacious & highly tolerable treatment option

### SYMPHONY-1 data at ASCO 2022

Combination showed encouraging activity in all relevant sub-populations

Acquisition also included first-in-class oral SETD2 inhibitor & portfolio of preclinical programs

# Onivyde



## Potential in 1L PDAC

### 1L data to be presented at ASCO GI, San Francisco

**Potential to expand Onivyde's peak-sales potential**

Current label:  
2L PDAC

**Onivyde regimen**

Statistically significant & clinically meaningful improvement in overall survival

**Trial met key secondary endpoint of progression-free survival**

A safety profile consistent with the previous trial

**1L duration of treatment & treated patients: U.S.<sup>1</sup>**

~5-6 months

~28,000 patients

**Leveraging Ipsen's existing in-market presence & building on the commitment to Oncology**

# Conclusion

*Successfully executing on our strategy*

## DELIVERING STRONG RESULTS



Strong progress on the four strategic pillars

Growth platforms performing well

Potential launches to drive further strong results

## FOCUSING ON EXTERNAL INNOVATION



Significant firepower

Adding pipeline assets; expanding the scope in Rare Disease

Momentum for further external-innovation transactions

## ADVANCING THE PIPELINE



Number of assets & trials

Opportunities across the three therapy areas

Several near-term milestones



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A photograph of an elderly woman with short grey hair and a young woman in blue scrubs smiling and holding hands in a brightly lit room. The elderly woman is on the left, wearing a green cardigan over a white shirt. The young woman is on the right, wearing blue scrubs over a white long-sleeved shirt. They are both smiling and looking at each other. The background is a blurred indoor setting with large windows.

# APPENDIX

# Oncology

## Key ongoing clinical-trial highlights

TRIAL	POPULATION	PATIENTS	DESIGN	PRIMARY ENDPOINT(S)	STATUS
<b>Cabometyx CONTACT-01</b> Phase III NCT04471428	2L NSCLC	<b>366</b> 	Docetaxel or Cabometyx + atezolizumab	OS	Primary endpoint not met
<b>Cabometyx CONTACT-02</b> Phase III NCT04446117	2L mCRPC	<b>580</b> 	Second novel hormonal therapy (abiraterone & prednisone or enzalutamide) or Cabometyx + atezolizumab	OS, PFS	Recruiting
<b>Onivyde NAPOLI-3</b> Phase III NCT04083235	1L PDAC	<b>770</b> 	Nab-paclitaxel + gemcitabine or Onivyde + 5-FU/LV + oxaliplatin	OS	Primary endpoint met  ASCO GI presentation Jan 2023

2L: second line; **NSCLC**: non-small cell lung cancer; **OS**: overall survival; **mCRPC**: metastatic castration-resistant prostate cancer; **PFS**: progression-free survival; **1L**: first line; **PDAC**: pancreatic ductal adenocarcinoma.

# Oncology

## Key ongoing clinical-trial highlights

TRIAL	POPULATION	PATIENTS	DESIGN	PRIMARY ENDPOINT(S)	STATUS
<b>Tazverik SYMPHONY-1</b> Phase III NCT04224493	R/R FL: following at least one prior systemic chemotherapy, immunotherapy, or chemoimmunotherapy	<b>540</b> 	Placebo + R <sup>2</sup> or Tazverik + R <sup>2</sup>	PFS	Recruiting
<b>Tazverik ARIA</b> Phase Ib/II NCT05205252	R/R hematologic malignancies	<b>156</b> 	Tazverik in various combinations: multi-cohort	Phase Ib: dosing, safety Phase II: ORR	Recruiting
<b>IPN60210</b> Phase I/Ib NCT05121103	R/R multiple myeloma and R/R DLBCL	<b>96</b> 	IPN60210	Treatment-emergent adverse events, dosing & ORR	Recruiting
<b>Tazverik CELLO-1</b> Phase Ib/II NCT04179864	mCRPC: patients who have not received chemotherapy	<b>104</b> 	Enzalutamide + Tazverik or abiraterone/prednisone + Tazverik	Phase Ib: dosing, safety  Phase II: rPFS Tazverik + enzalutamide	Recruiting

R/R: relapsed/refractory; FL: follicular lymphoma; R<sup>2</sup>: lenalidomide + rituximab; PFS: progression-free survival; ORR: objective response rate; DLBCL: diffuse large B-cell lymphoma; mCRPC: metastatic castration-resistant prostate cancer; rPFS: radiographic progression-free survival.

# Rare Disease

## Key ongoing clinical-trial highlights

TRIAL	POPULATION	PATIENTS	DESIGN	PRIMARY ENDPOINT(S)	STATUS
<b>Palovarotene</b> <b>MOVE</b> Phase III NCT03312634	FOP (chronic)	107 	Palovarotene - 5mg QD and upon flare-up, 20mg QD for 28 days, followed by 10mg for 56 days	Annualized change in new HO volume	E.U. regulatory decision anticipated H1 2023 U.S.: CRL issued Q4 2022
<b>Fidrisertib</b> <b>FALKON</b> Phase II NCT05039515	FOP (chronic)	~90 	Placebo or two dosing regimens of fidrisertib	Annualized change in new HO volume and safety	First patient commenced dosing Q1 2022
<b>Elafibranor</b> <b>ELATIVE</b> Phase III NCT04526665	2L PBC	161 	Placebo or elafibranor	Response to treatment defined as ALP < 1.67 x ULN and total bilirubin ≤ ULN and ALP decrease ≥ 15 percent	Recruitment completed Data anticipated H1 2023
<b>Elafibranor</b> <b>ELMWOOD</b> Phase II NCT05627362	PSC	60 	Placebo or elafibranor	Safety, significant changes in physical examination findings, laboratory parameters, vital signs, electrocardiogram readings	Initiating

# Neuroscience

## Key ongoing clinical-trial highlights

TRIAL	POPULATION	PATIENTS	DESIGN	PRIMARY ENDPOINT(S)	STATUS
<b>Mesdopetam</b> Phase IIb NCT04435431	Levodopa-induced dyskinesia in Parkinson's disease	<b>156</b> 	Mesdopetam or placebo	Change in average daily hours of ON-time <sup>1</sup> without troublesome dyskinesia	Recruitment completed Data anticipated H1 2023
<b>IPN59011 Ax LONG-SET</b> Phase I/II NCT04736745	Moderate to severe upper facial lines	<b>424</b> 	Dose escalation & dose finding versus Dysport or placebo	Safety	Terminated
<b>IPN10200 Ax LANTIC</b> Phase I/II NCT04821089	Moderate to severe upper facial lines	<b>424</b> 	Dose escalation & dose finding versus Dysport or placebo	Safety	Recruiting
<b>IPN10200 Tx LANTIMA</b> Phase I/II NCT04752774	Adult patients with upper limb spasticity	<b>209</b> 	Dose escalation & dose finding versus Dysport or placebo	Safety	Recruiting

**THANK  
YOU**

The background is a deep blue gradient. A complex network of thin white lines connects various points, creating a mesh-like structure. Several points in this network are highlighted with larger, glowing circles. Some of these circles are a bright cyan color, while others are a warm yellow. The overall effect is one of digital connectivity and data flow.

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