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# Syndax: A commercial-stage oncology company with two first-in-class medicines with practice-changing and billion-dollar potential



## Revuforj® (revumenib)

- First and only FDA-approved menin inhibitor
- Only targeted therapy for KMT2A translocations
- Launched in the U.S. in November 2024
- In development for mNPM1 AML and solid tumors

## Niktimvo™ (axatilimab-csfr)

- FDA approved in 3L chronic graft-versus-host disease (GVHD)
- U.S. launch, in partnership with Incyte, expected no later than early first quarter 2025
- In development for patients with newly diagnosed chronic GVHD, and IPF



**Delivered on major milestones** in 2024, including two FDA approvals and positive pivotal data in R/R mNPM1 AML



Well capitalized to support launch of two new medicines and **grow through label expansion and BD opportunities**



**Integrated commercial-stage organization**, with 225+ colleagues across R&D, clinical development, manufacturing, medical, commercial, and corporate

## Revuforj (revumenib)

First and Only Menin Inhibitor FDA-Approved to Treat Adult and Pediatric Patients with Relapsed or Refractory Acute Leukemia with a KMT2A Translocation

# There is an urgent need to improve treatment for R/R acute leukemia patients with a KMT2A translocation

## Disease Background

Rearrangements of the KMT2A gene (KMT2Ar), cause **~10% of acute leukemias**<sup>1</sup>

**>95% of KMT2Ar acute leukemia patients have a KMT2A translocation**, a type of rearrangement that occurs when part of one chromosome breaks and fuses to another<sup>2</sup>

KMT2Ar acute leukemia is associated with a **very poor prognosis and high rates of resistance and relapse**<sup>1,3</sup>

**5%** **historic complete remission (CR) rate** after  $\geq 3$  lines of conventional therapy<sup>3</sup>

**2.4 month** **median overall survival** after  $\geq 3$  lines of conventional therapy<sup>3</sup>



# Overview of Revuforj U.S. Prescribing Information

## Indication:

- Revuforj is a menin inhibitor indicated for the treatment of relapsed or refractory acute leukemia with a lysine methyltransferase 2A gene (KMT2A) translocation in adult and pediatric patients one year and older

## Dosage Forms & Strengths:

- Tablets: 25 mg, 110 mg, and 160 mg

## Dosage & Administration:

- Administered orally, twice daily
- Recommended dosage varies by patient weight and concomitant use of strong CYP3A4 inhibitors



**First and only FDA-approved menin inhibitor**

# Baseline demographics/characteristics from Revuforj Prescribing Information

*Across the Phase 1/2 AUGMENT-101 trial, Revuforj was studied in R/R acute leukemia patients with a KMT2A translocation*

Demographic and Disease Characteristics	Revuforj (N = 104)
<b>Age, median, years (range)</b>	37 (1, 79)
< 17 years old, n (%)	25 (24)
≥ 17 years old, n (%)	79 (76)
<b>Female, n (%)</b>	67 (64)
<b>AML/ALL/MPAL</b>	83%/15%/2%
<b>Disease status, n (%)</b>	
Primary refractory	22 (21)
Untreated relapse	21 (20)
Refractory relapse	61 (59)
<b>Number of previous regimens, median (range)</b>	2 (1, 11)
Prior stem cell transplantation, n (%)	46 (44)

Revuforj was studied in:

- Advanced, heavily pretreated patients
- A broad range of patients, including both adults and children with AML, ALL, or MPAL

# Efficacy summary from Revuforj Prescribing Information

Efficacy results from the Phase 1/2 AUGMENT-101 trial formed the basis for the approval of Revuforj

Endpoint	Revuforj N = 104
CR+CRh, n (%) 95% CI	22 (21.2) (13.8, 30.3)
Median duration of CR+CRh (months) 95% CI	6.4 (2.7, NE)
CR, n (%) 95% CI	13 (12.5) (6.8, 20.4)
Median duration of CR (months) 95% CI	4.3 (1.0, NE)
CRh, n (%) 95% CI	9 (8.7) (4.0, 15.8)
Median duration of CRh (months) 95% CI	6.4 (1.9, NE)

**1.9 months**  
median time to  
CR or CRh

**23% (24/104)** of  
patients underwent  
HSCT following  
treatment with  
Revuforj

# Safety overview from Revuforj Prescribing Information

The label includes safety data from 135 R/R acute leukemia patients (104 adult and 31 pediatric) with a KMT2A translocation who were treated with Revuforj

## Boxed warning:

- Differentiation syndrome (DS)

## Contraindications:

- None

## Warnings & precautions:

- QTc interval prolongation
- Embryo-fetal toxicity

Revuforj (N=135)	
Adverse reactions leading to dose interruption	42%
Adverse reactions leading to dose reduction	10%
Adverse reactions leading to permanent discontinuation	12%

**Most common (≥20%) adverse reactions including lab abnormalities:** hemorrhage, nausea, phosphate increased, musculoskeletal pain, infection, aspartate aminotransferase increased, febrile neutropenia, alanine aminotransferase increased, parathyroid hormone intact increased, bacterial infection, diarrhea, differentiation syndrome, electrocardiogram QT prolonged, phosphate decreased, triglycerides increased, potassium decreased, decreased appetite, constipation, edema, viral infection, fatigue, and alkaline phosphatase increased.

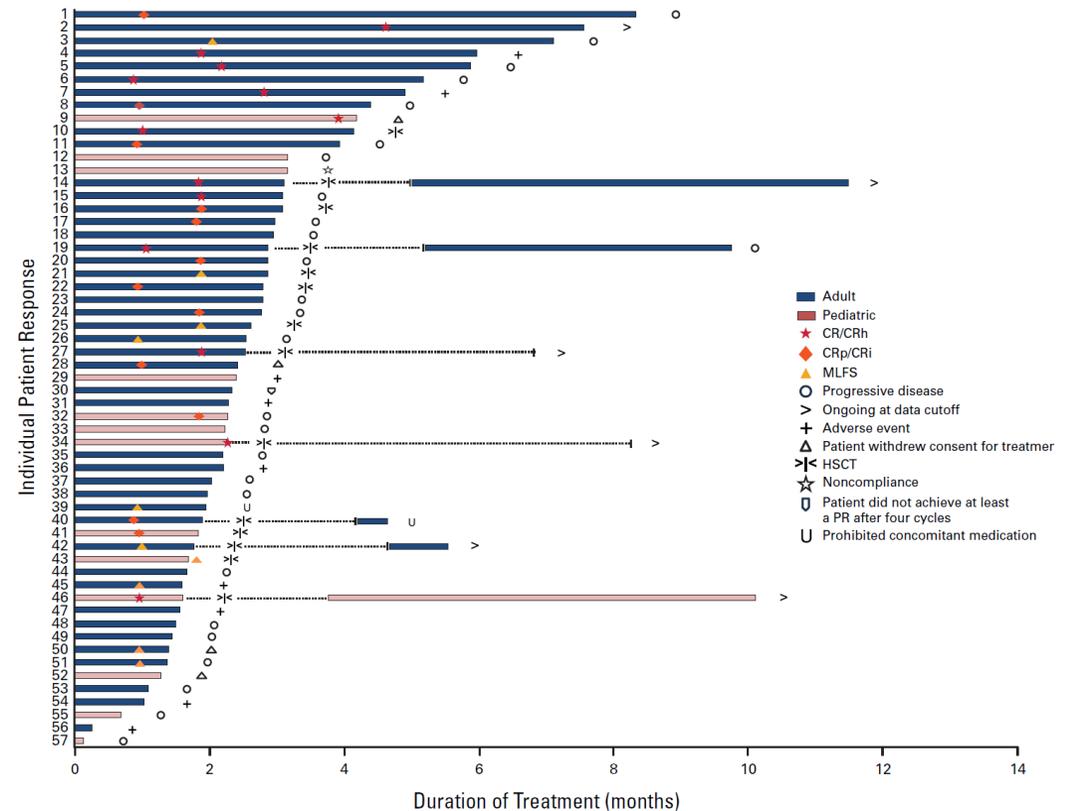
**Drug interactions:** Strong CYP3A4 inhibitors, strong or moderate CYP3A4 inducers, or QTc prolonging drugs

# JCO publication highlights rates of ORR, MRD negativity and HSCT in R/R acute leukemia patients with a KMT2A translocation who received Revuforj

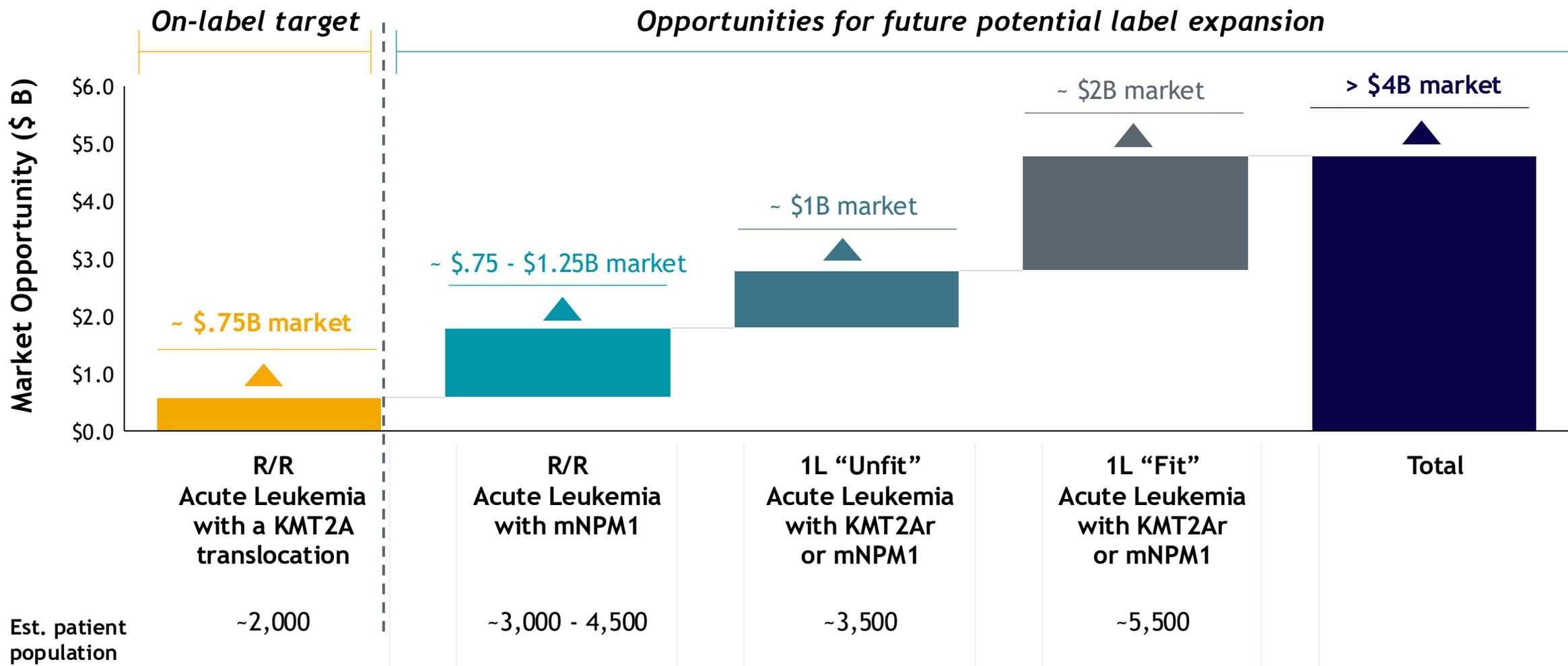
Publication includes data from the 57 efficacy evaluable patients in the pre-specified Phase 2 AUGMENT-101 trial interim analysis

## Ph 2 Interim Analysis Efficacy Population (n=57)

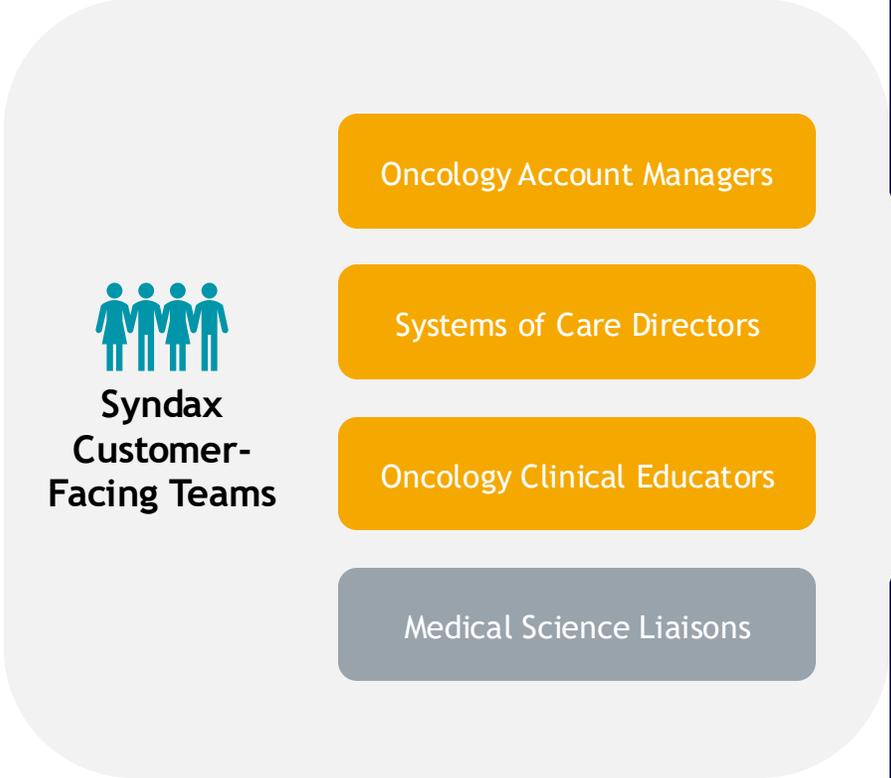
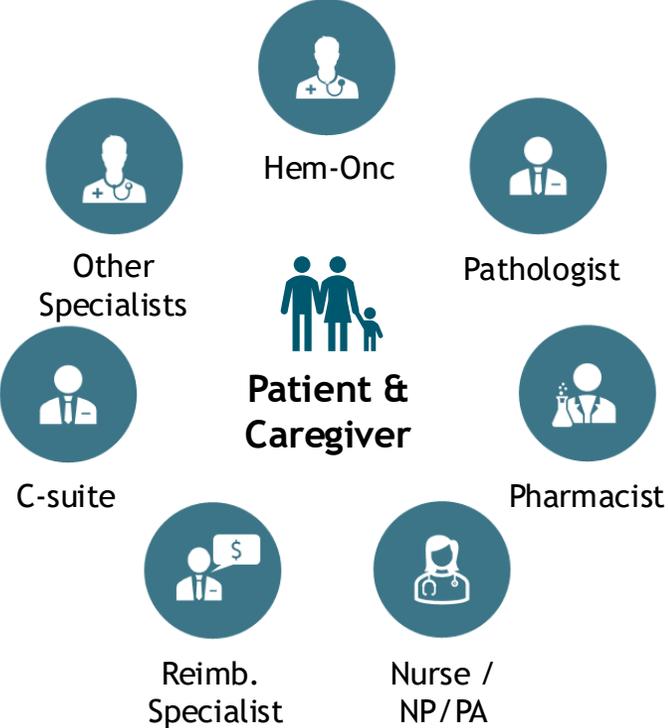
Overall response rate (ORR)	36 (63%)
CR + CRh	13 (23%)
CRc	25 (44%)
<b>MRD negative rate within evaluable patients</b>	
Within CR + CRh	7/10 (70%)
Within CRc	15/22 (68%)
Proceeded to HSCT	14/36 (39%)
Resumed revumenib post-HSCT	7/14 (50%)



# Initial Revuforj indication in R/R acute leukemia with a KMT2A translocation represents a significant market opportunity with potential for label expansion



# Highly-experienced customer facing teams are built to drive best-in-class patient and customer experience



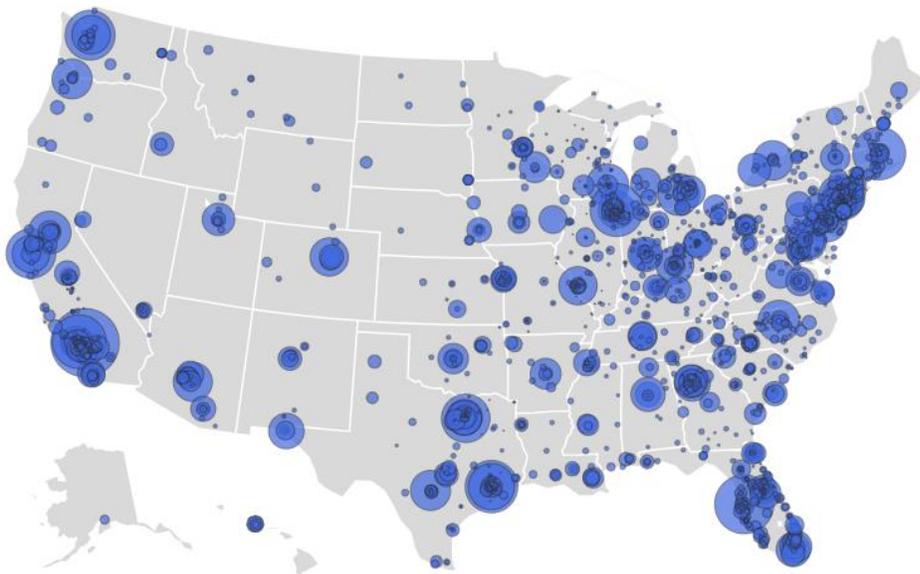
~50 highly experienced professionals will support Revuforj launch

- 22 years of average experience, primarily in hem/onc
- 6 product launches on average
- Strong pre-existing relationships

Multi-disciplinary teams will support key stakeholders across the patient journey with advanced data/analytics leveraged to drive patient identification and HCP targeting

# Field teams will target ~2,000 accounts where 98%+ of KMT2Ar patients receive treatment

~200 accounts are estimated to represent 2/3 of the opportunity



Note: Bubble size corresponds to prevalent AML+ALL patients

Key pre-launch activities have been completed, setting strong foundation for launch

✓ Drive Syndax and disease state/mechanism of disease awareness with target accounts

✓ Profile accounts to understand patient journey and treatment workflows

✓ Identify/validate key stakeholders for future product discussions

✓ Tier/prioritize accounts based on patient volumes

# Syndax is uniquely positioned to establish a successful menin franchise



First and only FDA-approved menin inhibitor enables significant **first-to-market advantages**



Urgent unmet need in R/R KMT2Ar acute leukemia expected to drive **rapid uptake**

*Revuforj is positioned for long-term success*



Development program included **both adults and pediatrics**, supporting a broad patient population in first indication



Opportunity to pursue future label expansion, with **promising clinical data across the broadest population** to date for a menin inhibitor



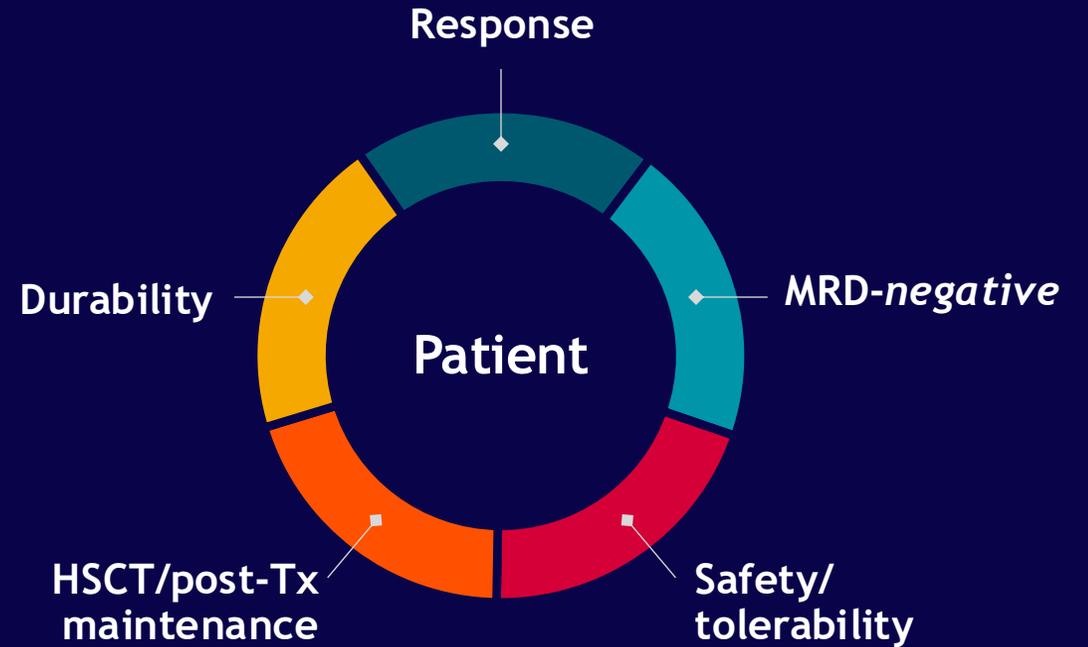
Opportunity for a unique launch trajectory, with potential for a **fast-follow indication in R/R mNPM1 AML** leveraging sNDA pathway and established commercial infrastructure

# Revumenib - Development Pipeline

# Revumenib has practice-changing potential in R/R mNPM1 AML

Met the primary endpoint in pivotal AUGMENT-101 cohort enrolling patients with NPM1 mutations

- Robust remission rates in heavily pre-treated population
- Remissions were notably deep and meaningfully durable
- A number of responders proceeded to HSCT, with the majority resuming revumenib post-transplant
- Favorable safety and tolerability supports continued evaluation of use in maintenance and in combination
- Results highlight consistency of revumenib's compelling clinical profile



Revumenib delivers on key metrics that address the needs of patients and drive physician utilization

# There is an urgent need for new treatment options for R/R mNPM1 AML patients

## mNPM1 AML DISEASE BACKGROUND

NPM1 mutations are the most **common genetic alterations in AML**

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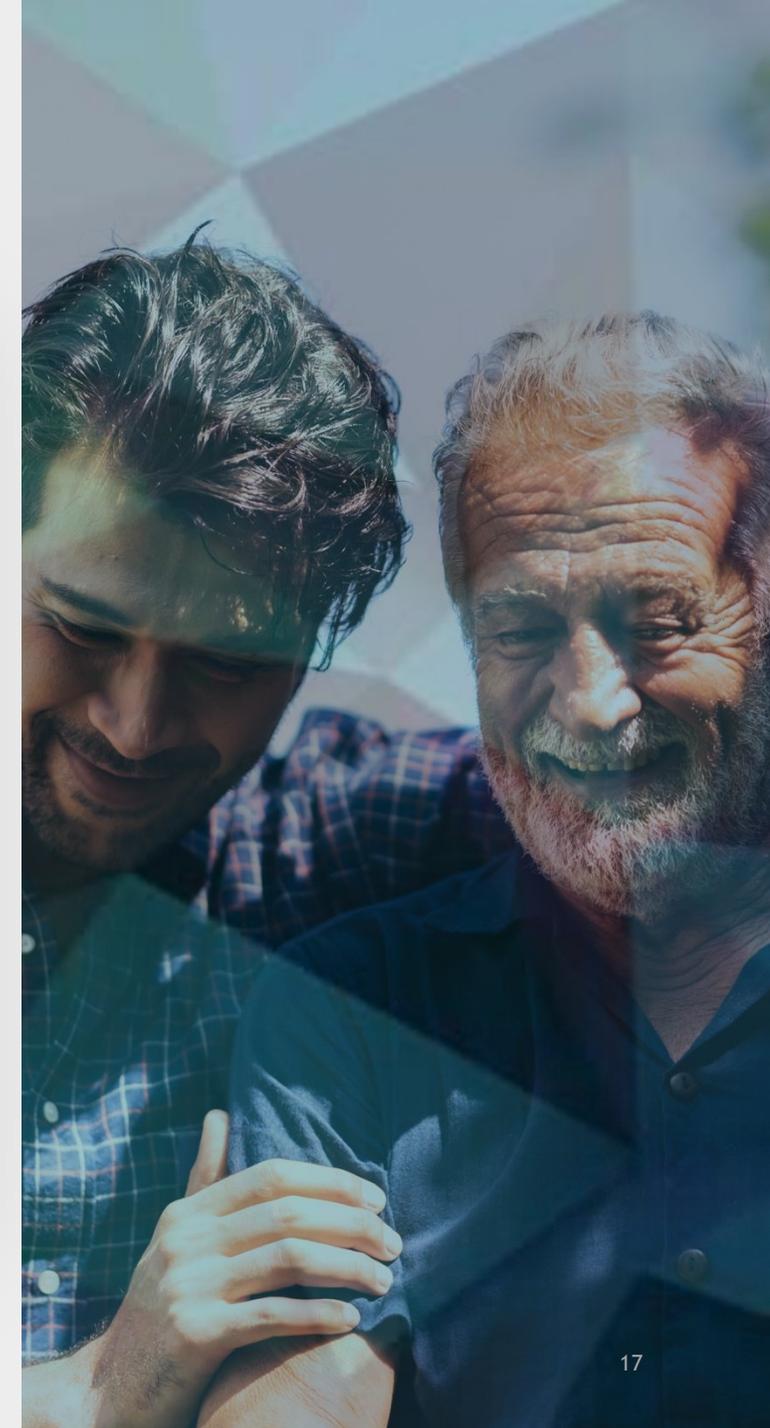
**~30%** of AML patients have NPM1 mutations

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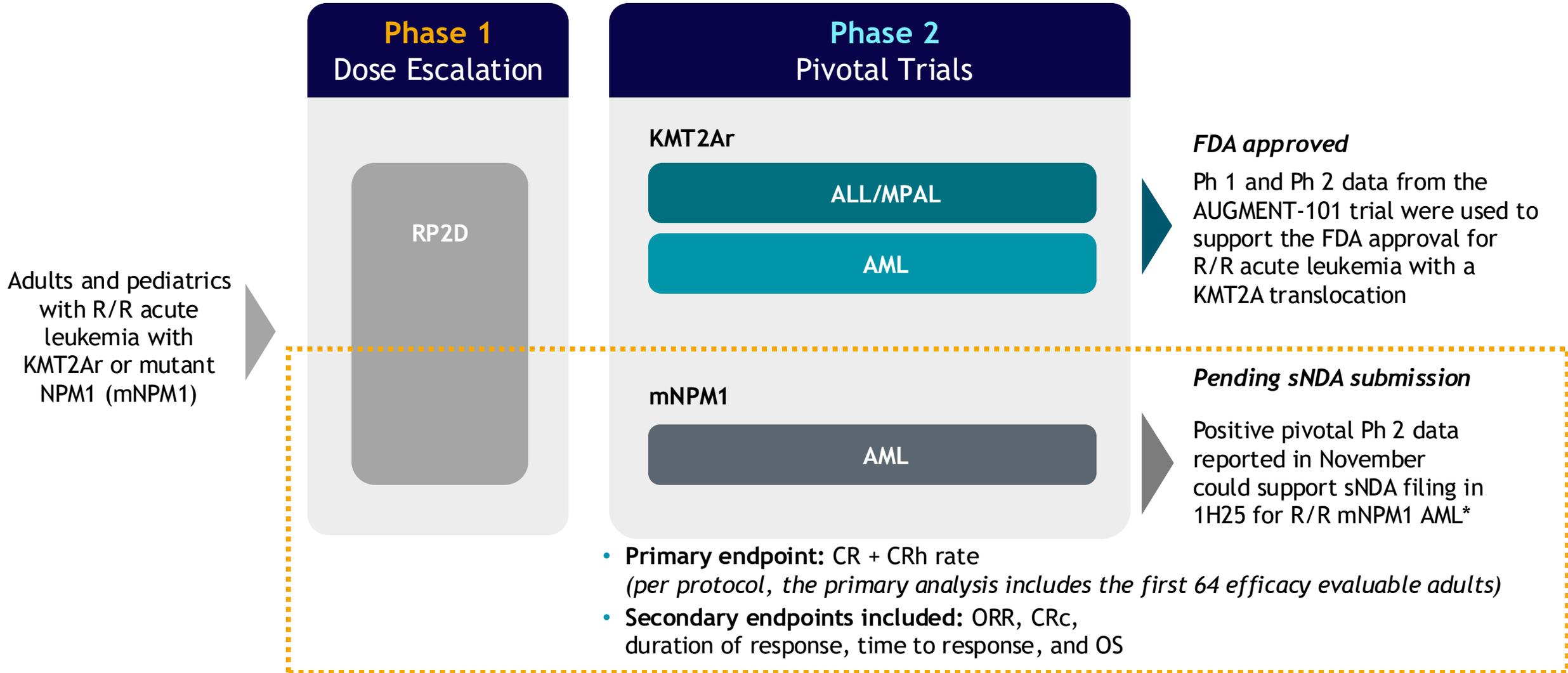
On average, mNPM1 patients are **older, and less fit for HSCT** than KMT2Ar patients

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R/R mNPM1 AML patients have a **poor prognosis and high unmet need**



# AUGMENT-101: A Phase 1/2 trial of revumenib monotherapy in R/R mNPM1 and KMT2Ar acute leukemia



# AUGMENT-101 baseline characteristics in Phase 2 cohort of patients with R/R mNPM1 AML

Baseline Characteristics	Protocol-Defined Adult Efficacy Population N = 64	Safety Population N = 84
<b>Age, years, median (range)</b>	65 (19, 84)	63 (11, 84)
≥ 18 to <65, n (%)	31 (48)	42 (50)
≥ 65, n (%)	33 (52)	41 (49)
<b>Female, n (%)</b>	38 (59)	50 (60)
<b>Baseline co-mutations of interest, n (%)</b>		
FLT3-ITD	22 (34)	26 (31)
FLT3-TKD	4 (6)	6 (7)
IDH1	8 (13)	11 (13)
IDH2	8 (13)	10 (12)
<b>Disease Status at Baseline, n (%)</b>		
Primary refractory (persistent leukemia following induction chemotherapy)	5 (8)	7 (8)
Refractory relapse (unresponsive to most recent salvage treatment)	35 (55)	41 (49)
<b>Prior lines of therapy, median (range)</b>	2 (1, 7)	2 (1, 7)
≥3 lines, n (%)	23 (36)	29 (35)
Prior venetoclax, n (%)	48 (75)	62 (74)
Prior HSCT, n (%)	14 (22)	20 (24)

Patients were significantly older than R/R KMT2Ar cohort

75% had prior venetoclax exposure in efficacy population

36% received revumenib in the 4L or later in efficacy population

# Revumenib demonstrated compelling efficacy in R/R mNPM1 AML patients in Ph 2 portion of AUGMENT-101

Best Response, n (%)	Protocol-Defined Adult Efficacy Population N = 64	Post-Hoc Efficacy Evaluable Adult + Pediatric Population <sup>1</sup> N = 77
CR/CRh	15 (23%) (95% CI: 14%, 36%) <i>one-sided p-value: 0.0014</i>	20 (26%) (95% CI: 17%, 37%)
CR	12 (19%)	16 (21%)
CRh	3 (5%)	4 (5%)
MRD negative CR/CRh*	9/14 (64%)	12/19 (63%)
Median duration of CR/CRh	4.7 months	4.7 months
Overall Response Rate (ORR)	30 (47%)	37 (48%)
Composite complete remission (CRc)	19 (30%)	25 (32%)
MRD negative CRc*	10/17 (59%)	13/23 (57%)

ORR = CR+CRh+CRp+CRi+MLFS+PR; CRc = CR+CRh+CRp+CRi; \* Not all patients had MRD status reported. Totals may not sum due to rounding.

Data cutoff of September 18, 2024; 1. Post-hoc efficacy evaluable population consists of all Phase 2 R/R mNPM1 AML patients with central confirmation of NPM1 mutation and blast counts >5% measured within 28 days prior to treatment. Of the 84 patients in the Phase 2 R/R mNPM1 safety population, 7 patients did not meet the efficacy evaluable criteria and were therefore excluded from the post-hoc efficacy analysis.

Results in Ph 2 protocol-defined efficacy population are consistent with post-hoc analysis of all enrolled Ph 2 mNPM1 patients who met efficacy evaluable criteria

~50% of patients achieved an overall response in heavily pre-treated population

Deep, meaningfully durable CR/CRh responses

17% (5/30) of responders went to HSCT following revumenib, in the protocol-defined efficacy population

# Safety results from Phase 2 R/R mNPM1 AML cohort in the AUGMENT-101 trial support favorable revumenib safety and tolerability profile

Grade $\geq$ 3 Treatment-Related Adverse Events [TRAEs] ( $\geq$ 5% of patients)	Safety Population N = 84
Patients with Grade $\geq$ 3 TRAE	50 (60%)
Electrocardiogram QT prolonged	18 (21%) (Gr 3: 19%   Gr 4: 2%)
Anemia	12 (14%)
Febrile neutropenia	11 (13%)
Differentiation syndrome	11 (13%) (Gr 3: 11%   Gr 4: 2%)
Platelet count decreased	9 (11%)
Thrombocytopenia	8 (10%)
White blood cell count decreased	7 (8%)
Neutrophil count decreased	6 (7%)

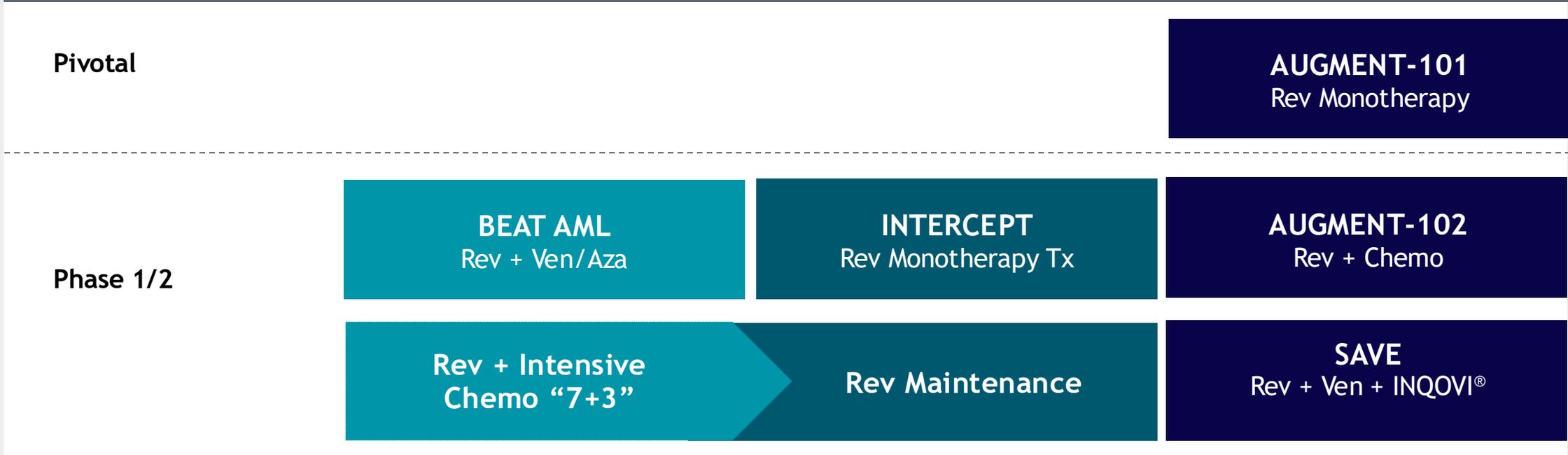
- Safety results in this older, heavily pre-treated population were consistent with previously reported data
- Low rate of treatment-related discontinuations (5%)
- Most common adverse events observed are largely characteristic of symptoms experienced by patients undergoing treatment for AML

# Multiple ongoing and planned revumenib clinical trials support potential label expansion and franchise opportunity

KMT2Ar & mNPM1  
acute leukemia  
treatment paradigm



Revumenib clinical development program (acute leukemia with a KMT2Ar or NPM1 mutation) - ongoing trials



# SAVE: Revumenib-ven/HMA combo in R/R AML resulted in high rates of remission, MRD negativity & HSCT

Updated data presented at ASH 2024 (abstract #216)

## Baseline Characteristics (N=33)

Age, years, median (range)	35 (12-81)
KMT2Ar/mNPM1/NUP98r, n	16/12/5
Prior lines of therapy, median (range)	3 (1,5)
Prior venetoclax	19 (58%)
Prior HSCT	12 (36%)
Prior menin inhibitor	2 (6%)

## Safety Highlights

- Combination was generally well tolerated
- The most common (>20%) Grade ≥3 treatment-emergent adverse events were febrile neutropenia and lung infection

## Best Response (N=33)

ORR	27 (82%)
CR+CRh	16 (48%)
CR	13 (39%)
CRh	3 (9%)
MRD negative responders	17/26 (65%)*
MRD negative CR/CRh	14/16 (88%)
Median duration of CR/CRh response	Not reached
Proceeded to HSCT	13/33 (39%)
Resumed revumenib post-HSCT	7/13 (54%)

ORR= CR+CRh+CRp+PR+MLFS; \*One responding pt had inadequate MRD by MFC.

With median follow-up of 9.3 months, the 6-month OS was 68%; median OS was not reached

SAVE is now enrolling a cohort of newly diagnosed patients who are ineligible for intensive chemotherapy

# Updated BEAT AML data show revumenib's potential to enhance ven/aza combo in frontline mNPM1 or KMT2Ar AML

## Summary of Enrolled Patients (N=46)

Genetics, n	mNPM1: 37 KMT2Ar: 9
Age, years, median (range)	71 (60-92)

## Summary of Outcomes in Efficacy Evaluable Population (n=37)

<b>Composite CR (CRc)</b>	<b>35/37 (95%)</b>
<b>Overall response rate (ORR)</b>	<b>37/37 (100%)</b>
MRD negative	35/37 (95%)
Proceeded to HSCT	10/37 (27%)

CRc = CR+CRh+CRp+CRi; ORR = CRc+MLFS+PR

## Safety Highlights

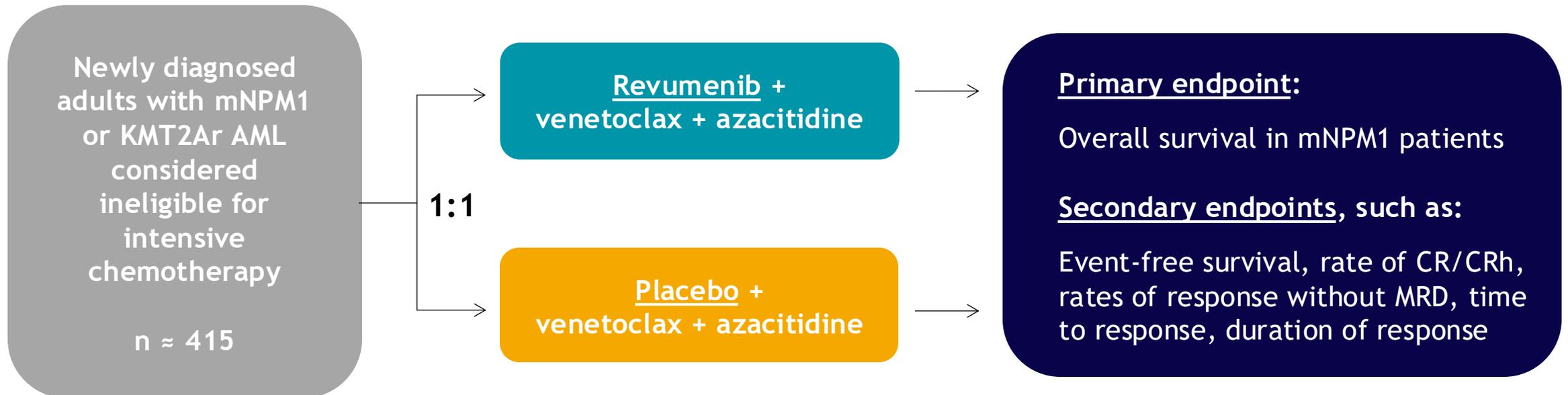
- Revumenib was generally well tolerated at both the 113 mg and 163 mg q12h dose in combination with ven/aza
- No new or increased safety signals observed when revumenib was included in this triplet combination
- DS and QTc prolongation were self-limiting and did not cause any discontinuations

**Enrollment in the expansion cohort is ongoing at both dose levels**

**Syndax plans to initiate pivotal trial with this frontline combo by YE24**

# Pivotal frontline trial of revumenib + ven/aza on track to initiate by YE24

*Frontline triplet will be studied in a randomized, double-blind, placebo-controlled, clinical trial in collaboration with the HOVON network*



## **Niktimvo (axatilimab-csfr) - anti-CSF-1R antibody**

FDA approved for treatment of certain patients with chronic graft-versus-host disease in 2024 with opportunities for potential label expansion

# Niktimvo (axatilimab-csfr) is now FDA approved



- ✓ **FDA approved** for treatment of chronic GVHD after failure of at least two prior lines of systemic therapy in adult and pediatric patients weighing at least 40 kg
- ✓ Included in **NCCN Guidelines**<sup>®</sup>
- ✓ Preparing for **U.S. launch** no later than early 1Q25

## Syndax and Incyte are co-commercializing Niktimvo in the U.S.



### *Within U.S.*

30% sales effort  
50% profit

### *Outside U.S.*

Double-digit royalties  
and milestones

70% sales effort  
50% profit

Exclusive rights to  
commercialization

## Niktimvo clinical development programs

### Underway:

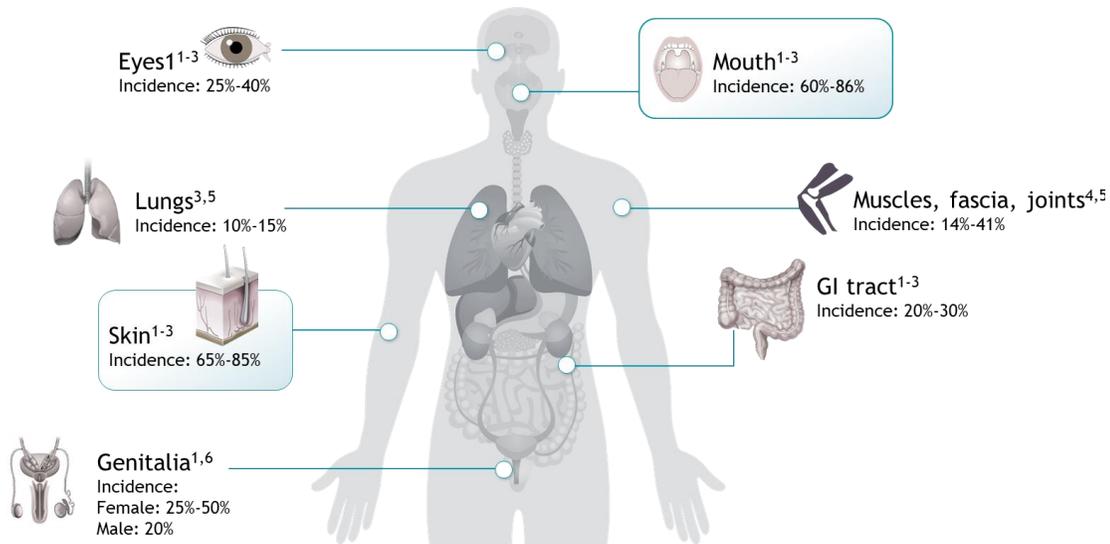
- Ph 2 MAXPIRe trial in idiopathic pulmonary fibrosis (IPF)
- Ph 2 frontline combo trial with Jakafi<sup>®</sup> in cGVHD

### In preparation:

- Ph 3 frontline combo trial with steroids in cGVHD

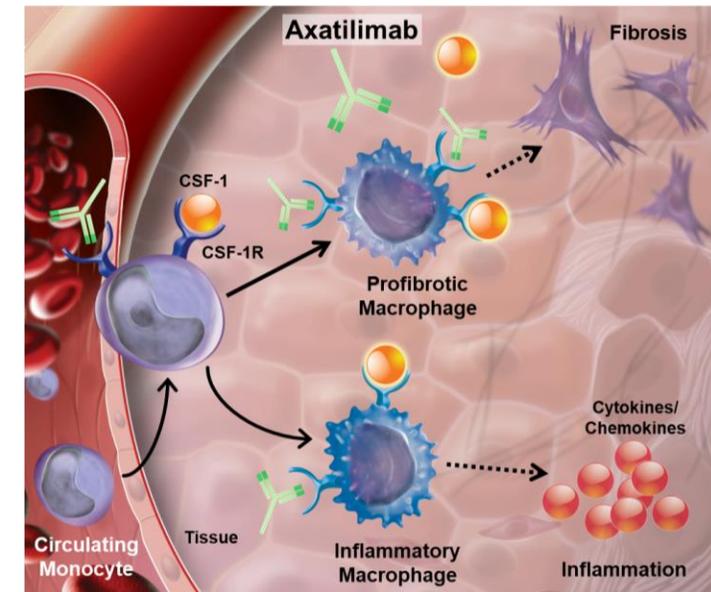
# There is a significant need for a new therapeutic approach to cGVHD, a debilitating and difficult to treat disease

cGVHD impacts multiple organ systems, drives significant morbidity and impairs quality of life



Complete responses are rare, and many organs respond poorly to available therapies; nearly 50% of patients progress to third line treatments<sup>1</sup>

Niktimvo is the first approved anti-CSF-1R antibody targeting the drivers of inflammation and fibrosis in cGVHD



Blocking CSF-1R with Niktimvo reduces the levels of proinflammatory and profibrotic monocytes and monocyte-derived macrophages

# Overview of Niktimvo (axatilimab-csfr) U.S. prescribing info

## INDICATION

- Niktimvo is indicated for the treatment of chronic graft-versus-host disease (cGVHD) after failure of at least two prior lines of systemic therapy in adult and pediatric patients weighing at least 40 kg
- 

## DOSAGE & ADMINISTRATION

- The recommended dosage of Niktimvo is 0.3 mg/kg (maximum 35 mg) every 2 weeks in adult and pediatric patients weighing at least 40 kg
- Administered as an intravenous infusion over 30 minutes

# Niktimvo FDA approval was based on AGAVE-201 trial data



A randomized, open-label, multicenter study (121 study sites, 16 countries)

## Key eligibility criteria:

- Adult and pediatric participants with recurrent or refractory active cGVHD whose disease had progressed after  $\geq 2$  prior therapies
- Concomitant use of corticosteroids, calcineurin inhibitors, or mTOR inhibitors was allowed; no additional systemic cGVHD therapy was allowed

## Primary endpoint:

- Overall response rate (ORR) by Cycle 7 (28-day cycles) Day 1, including complete response or partial response according to the NIH 2014 Consensus Criteria<sup>1</sup>

## Secondary and exploratory endpoints:

- Improvement in mLSS ( $\geq 7$  points)
- Organ-specific response rates, DOR, FFS, OS
- Safety

Of the three doses evaluated<sup>2</sup>, 0.3 mg/kg IV every 2 weeks was selected as the recommended therapeutic dose

# AGAVE-201 enrolled advanced, heavily pre-treated cGVHD patients

Demographics & Baseline Characteristics of Patients with cGVHD	Niktimvo 0.3 mg/kg every 2 weeks (N=79)
Median age, years (range)	50 (7, 76)
Male, n (%)	46 (58%)
Race - white, n (%)	67 (85%)
Median time (range) from cGVHD diagnosis	47 months (4, 211)
≥ 4 organs involved, n (%)	45 (57%)
Severe cGVHD, n (%)	63 (80%)
Median (range) prior lines of therapy	4 (2, 12)
≥ 4 prior lines of treatment, n (%)	54 (69%)
Prior ruxolitinib, n (%)	57 (72%)
Prior ibrutinib, n (%)	27 (34%)
Prior belumosudil, n (%)	16 (20%)

Compared to the registrational population in the belumosudil trial<sup>1</sup>, AGAVE-201 enrolled a population with:

- Significantly longer median time since diagnosis (47 vs. 25 months)
- More severe cGVHD (80% vs 71%)
- More exposure to prior therapies (e.g., 72% vs 31% exposed to ruxolitinib)

# Summary from Niktimvo U.S. prescribing information

Efficacy results	Niktimvo 0.3 mg/kg every 2 weeks (N=79)
<b>Overall response rate through 6 months, n (%)</b> 95% CI	<b>59 (75%)</b> 64, 84
Median time to first response (range)	1.5 months (0.9-5.1)
Median duration of treatment (range)	10.3 months (0.5-28.6)

**60% of responders maintained a response for at least 12 months<sup>1</sup>**  
 (95% CI: 43, 74)

**56% of patients reported symptom improvements<sup>2</sup>**  
 (95% CI: 44, 67)

# Safety summary from Niktimvo U.S. prescribing information

## Boxed warning:

None

## Contraindications:

None

## Warnings & precautions:

Infusion-related reactions and embryo-fetal toxicity

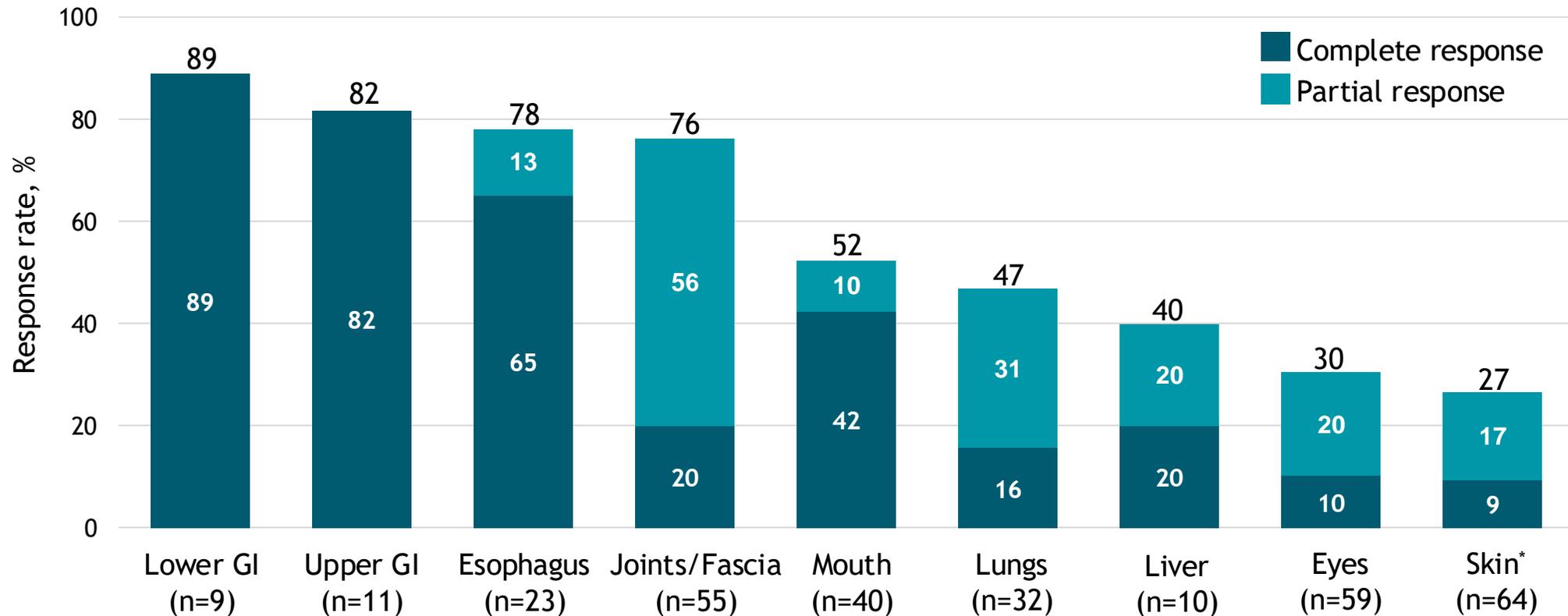
## Most common ( $\geq 15\%$ ) adverse reactions, including lab abnormalities:

Increased AST, infection (pathogen unspecified), increased ALT, decreased phosphate, decreased hemoglobin, viral infection, increased GGT, musculoskeletal pain, increased lipase, fatigue, increased amylase, increased calcium, increased CPK, increased ALP, nausea, headache, diarrhea, cough, bacterial infection, pyrexia, and dyspnea.

Niktimvo 0.3 mg/kg every 2 weeks (N=79)	
	% of patients
Serious adverse reactions	44%
Dose interruptions due to adverse reactions	44%
Dose reductions due to adverse reactions	8%
Permanent discontinuation due to adverse reactions	10%

# Niktimvo showed robust responses across all organs studied in the heavily pre-treated population enrolled in AGAVE-201

Niktimvo 0.3 mg/kg every two weeks



Responses were notable in fibrosis-dominated organs, including esophagus (78%), joints and fascia (76%), lung (47%), and skin (27%)

## Targeted Niktimvo launch strategy will be led by highly experienced team from Incyte and Syndax

- Incyte to lead commercialization and contribute 70% of sales effort, leveraging their leadership in GVHD and extensive pre-existing relationships
- Syndax to contribute 30% of sales effort, deploying its own highly experienced field force
  - Average of 22 years of experience, primarily in hematology/oncology, with an average of 6 product launches
  - Overlapping call point with Revuforj targets allows for commercial synergies

**At launch, Incyte & Syndax will prioritize top centers with the highest volumes and greatest opportunity for rapid uptake**



Robust stakeholder engagement and education is underway to support successful U.S. launch

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# Niktimvo has a significant opportunity in 3L cGVHD in the U.S., with opportunities for geographical and label expansion

2024



Beyond



Of the 17,000 U.S. chronic GVHD patients on treatment at any one time, we will target the ~6,500 progressing to later lines of treatment after at least two previous lines of treatment, consistent with our label

cGVHD  
(U.S.)  
~17,000<sup>1</sup>

cGVHD  
(W.W.)  
>35,000<sup>2</sup>

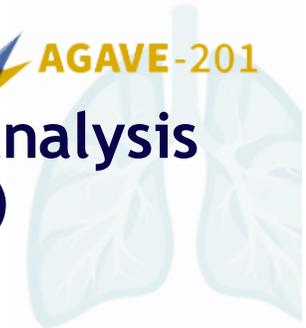
Fibrotic diseases,  
such as IPF  
~215,000 U.S.  
~335,000 W.W.<sup>3</sup>

Phase 2 IPF trial underway  
Evaluating other potential  
disease areas

1L chronic GVHD combination trials planned to initiate in 2H24 could support future potential label expansion

# Antifibrotic activity of axatilimab highlighted in AGAVE-201 subgroup analysis of patients with cGVHD related bronchiolitis obliterans syndrome (BOS)

Data presented at ERS 2024



**Rapid and robust BOS response rates despite inclusion of patients with severe BOS**

**Clinically meaningful improvements in symptoms of shortness of breath (SOB) at rest or with exertion**

Patients with BOS in AGAVE-201      0.3 mg/kg (n=32)      1mg/kg (n=41)      3 mg/kg (n=35)

**Characteristics**

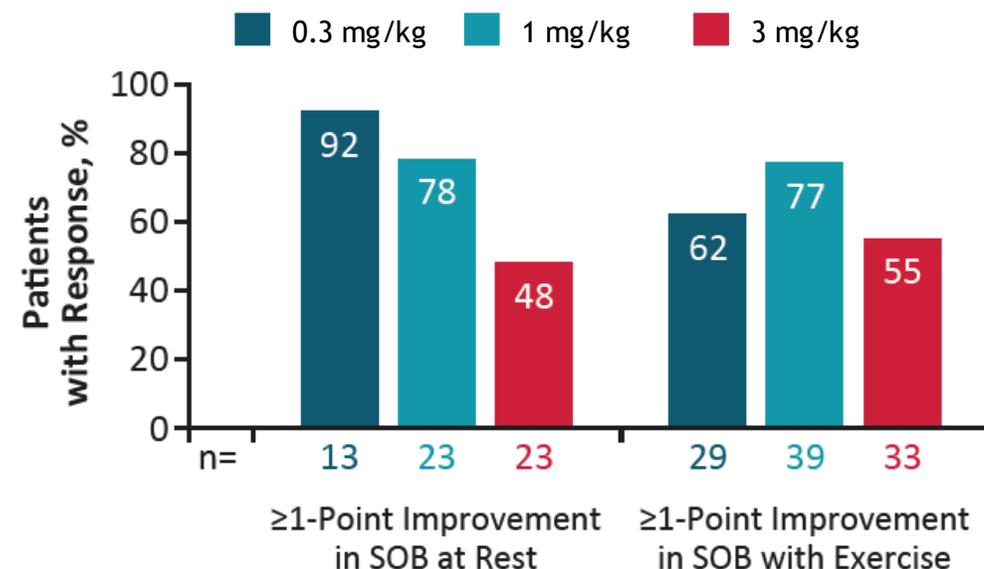
Number of prior systemic cGVHD therapies, median (range)	4 (2-10)	4 (2-10)	4 (2-12)
Lung involvement at baseline, n (%)	32 (100)	41 (100)	35 (100)
FEV <sub>1</sub> ≤39%	14 (47)	15 (42)	7 (26)
NIH cGVHD lung symptom score of 3	8 (25)	10 (26)	8 (23)

**Efficacy**

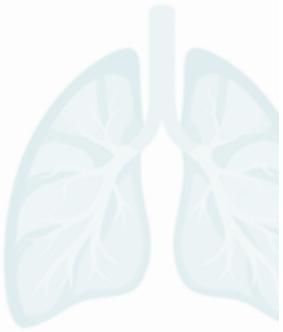
<b>BOS response, n (%)</b>	<b>15 (47)</b>	<b>14 (34)</b>	<b>13 (37)</b>
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**Median time to first BOS response was <3 months**

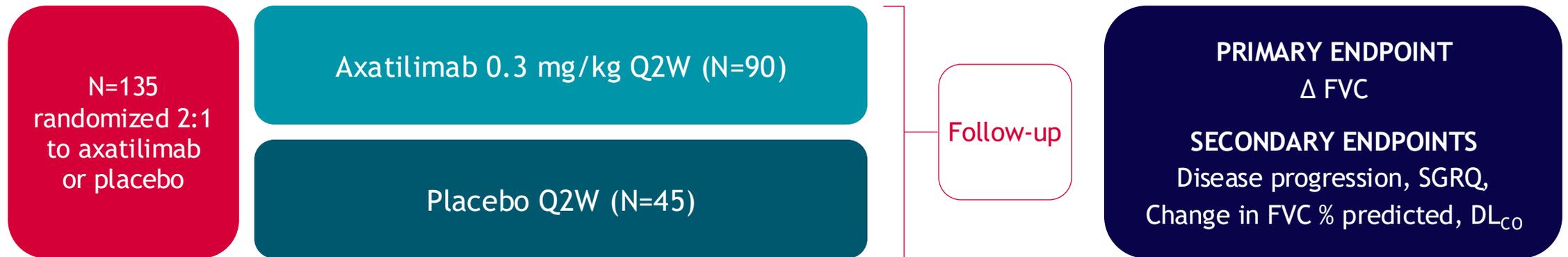
Patient reported improvements in SOB based on mLSS



# MAXPIRe Phase 2 trial of axatilimab in IPF is now enrolling patients with topline data anticipated in 2026



A 26-Week, Randomized, Double-Blind, Placebo-Controlled, Multi-Center Study to Evaluate the Efficacy, Safety, and Tolerability of Axatilimab in Subjects with Idiopathic Pulmonary Fibrosis (IPF)



Axatilimab's advancement into IPF supported by:

- Published preclinical and clinical rationale for CSF-1 pathway inhibition in IPF
- Clinical results from chronic GVHD trials showing the positive impact on lung fibrosis

# Corporate Highlights

# Financial highlights and financial guidance

Ticker	SNDX (NASDAQ)
Cash and equivalents <sup>†</sup> (30 September 2024)	\$399.6 M
Shares outstanding* (30 September 2024)	85.6 M
2024 Operating Expense Guidance	
	FY24
Research and development	\$245 - \$250 M
Total operating expenses <sup>^</sup>	\$365 - \$370 M

**Syndax expects that its cash, cash equivalents and marketable securities, together with the \$350 M from the sale of a portion of the Niktimvo royalty and anticipated product revenue and interest income, enables the company to reach profitability**



## Expected upcoming milestones

### Revuforj (revumenib)

Menin-KMT2A inhibition

- Initiation of pivotal combination trial with ven/aza in newly diagnosed mNPM1 or KMT2Ar acute leukemias by YE24
- Publish and present pivotal R/R mNPM1 AML data at a medical conference in 1H25
- sNDA filing in R/R mNPM1 AML in 1H25

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### Niktimvo (axatilimab-csfr)

CSF-1R inhibition

- Launch in refractory chronic GVHD no later than early first quarter 2025
- Chronic GVHD frontline combination trial with steroids in preparation
- Topline readout from Phase 2 IPF trial in 2026

