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# Strong 3Q25 commercial and portfolio execution position Syndax to reach profitability and be first to the frontline setting with amenin inhibitor

## Commercial Execution



**\$32.0M**  
net revenue

**+25% TRx**  
growth q/q



**\$45.8M**  
net revenue  
to INCY

**\$13.9M**  
collaboration  
revenue to SNDX

## Portfolio Advancements

**Revuforj added to NCCN  
Guidelines® for R/R NPM1m AML**  
on Sept 18, 2025

**Revuforj FDA-approved for  
second indication**  
on Oct 24, 2025

**23 Revuforj & Niktimvo  
abstracts accepted for  
presentation at ASH 2025**

**On the road to profitability, with growing product contributions, a robust balance sheet,  
and stable expense outlook**

# Strong 3Q25 Revuforj growth, even with a third of patients pausing Tx to proceed to stem cell transplant



	3Q25	Cumulative since launch
Net revenue	\$32M +12% q/q growth	\$88M
TRx	~850 +25% q/q growth	~2,200
New patient starts	~250 +25% q/q growth	~750

Revuforj is positioned for long-term growth with building usage observed post-HSCT and expansion into R/R NPM1m AML

## 3Q25 patient insights

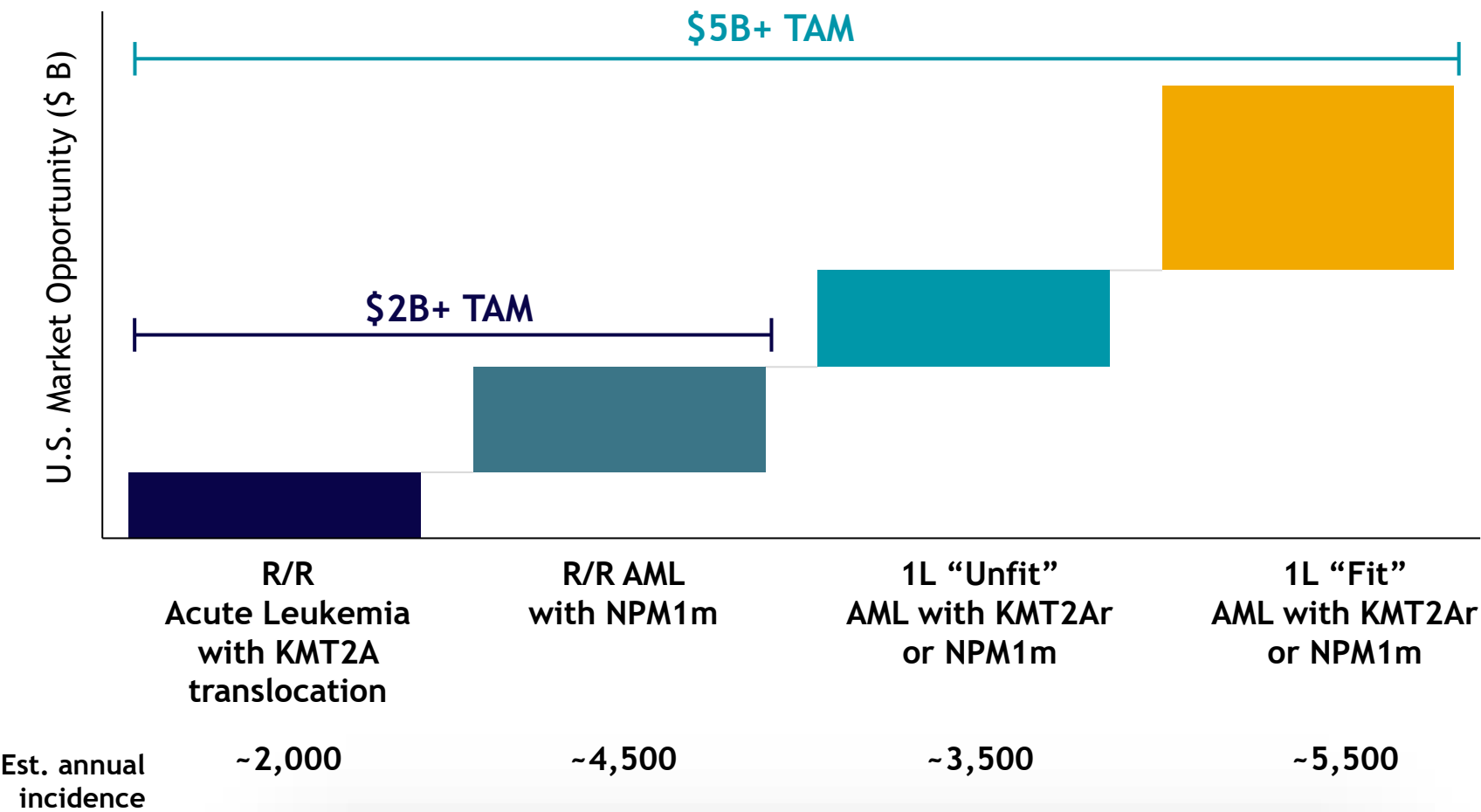
~90% of use in KMT2A

~70% of KMT2A use in 2L/3L setting

~33% of KMT2A pts. proceed to HSCT

~35-40% of KMT2A pts. resume Revuforj post-HSCT

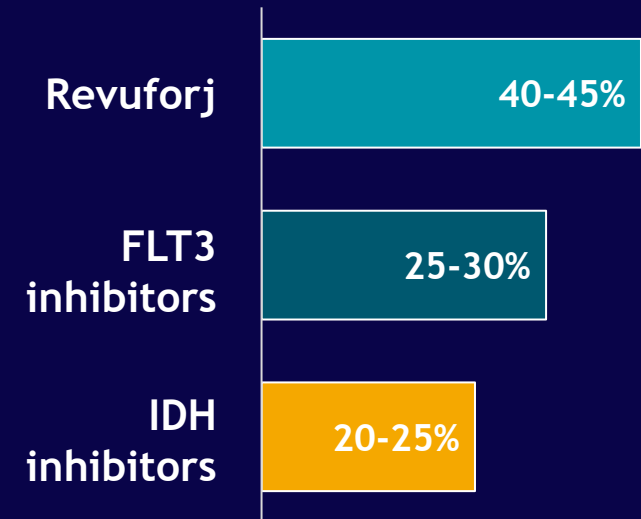
# Second Revuforj indication unlocks \$2B U.S. market opportunity in R/R acute leukemia alone



*NPM1 mutations and KMT2A translocations are routinely tested for, enabling efficient patient identification*

With the *largest addressable population* and anticipated duration of therapy, Revuforj is poised to become the largest targeted AML therapy

## Addressable AML population



# Revuforj expansion into R/R NPM1m AML is well underway, leveraging solid foundation



Unmatched efficacy  
data across multiple  
patient subtypes



Strong prescriber base  
& HCP familiarity

*Competitive  
advantages*

Excellent payer support  
& reimbursement



Proven track record of  
delivering for patients



# Robust 3Q25 Niktimvo growth reflects rapid uptake across U.S. bone marrow transplant centers



	3Q25	Cumulative since launch
Net revenue to INCY	\$45.8M +27% q/q growth	\$96M
Collaboration revenue to SNDX	\$13.9M +48% q/q growth	\$23M
Infusions administered	~4,500	8,500
New patient starts	~400	1,100

## 3Q25 insights

80%

of pts. that started in Q1 remain on Tx

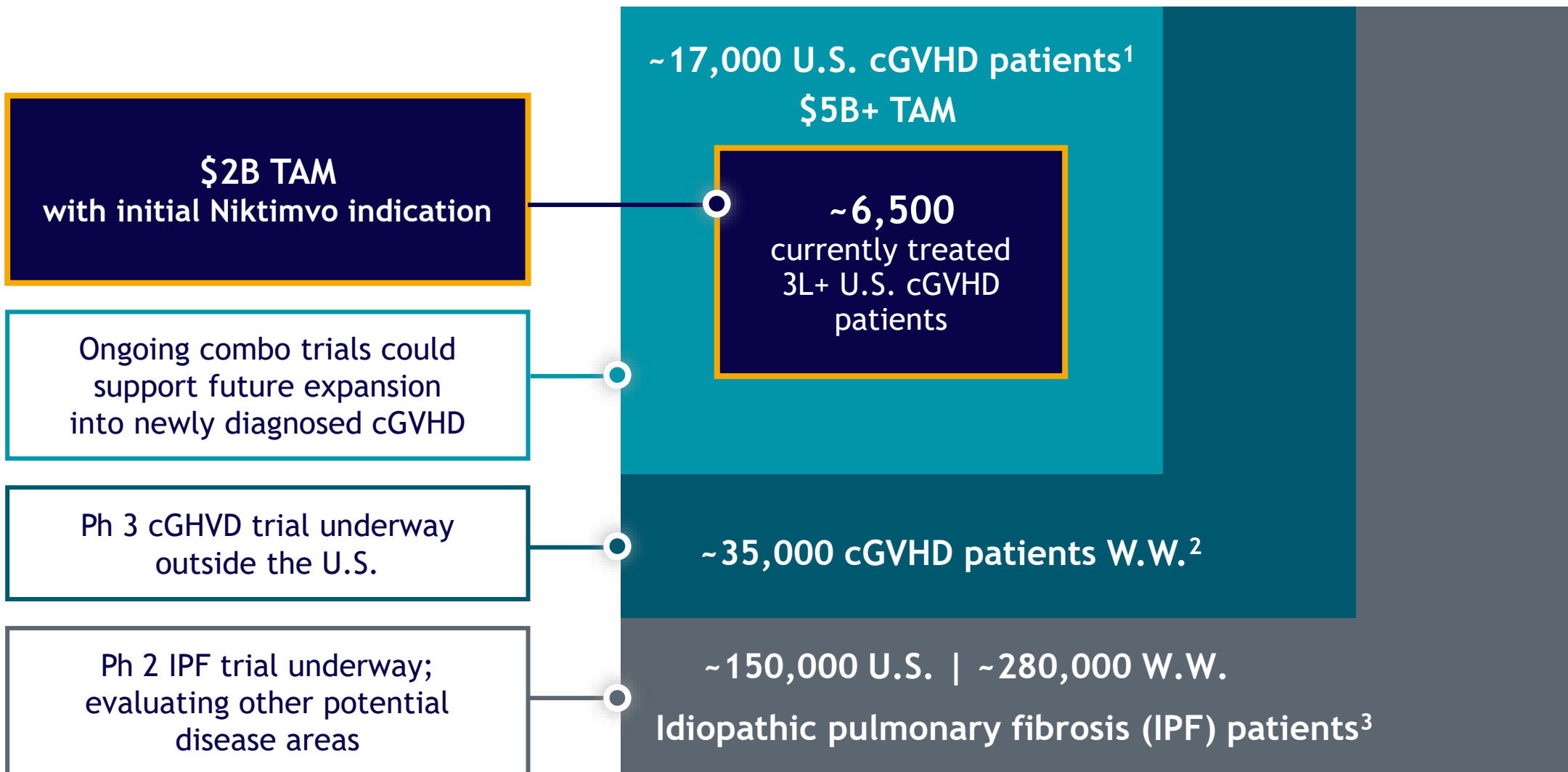
3L+

most usage in 4L; growing 3L use

90%

of U.S. bone marrow transplant centers have ordered

# Initial Niktimvo indication represents a \$2B U.S. market opportunity, with substantial opportunities for label and geographic expansion





# First Revuforj real-world evidence highlights favorable tolerability and excellent activity across genetic subtypes and settings

ASH 2025 abstract #3448

Real-world experience at Moffitt Cancer Center through July 2025

## Demographics/characteristics (n=18)

Age, median (range):	60 (23-79)
Prior lines, median (range):	3 (0-6)
Genetics, n (%)	9 (50%) KMT2Ar, 6 (33%) NPM1m, 3 (17%) NUP98r
Setting of therapy, n (%)	15 (83%) R/R, 2 (11%) 1L, 1 (6%) post-HSCT w/out prior rev
Rev usage, n (%)	14 (78%) in combination, 4 (22%) as monotherapy

### Safety overview (n=18)

- No AEs led to revumenib discontinuation
- Low rate of revumenib dose reductions: 11% (2/18)
- DS in 11% (2/18) of pts (1 G2 & 1 G3)
- QTc prolongation: 23% (3/13) G3 and 31% (4/13) G1/G2

## Early efficacy data (median follow up: 3.97 months)

Patients treated for morphologic disease/relapse (n=14)	
ORR	79% (11/14)
MRD negativity among responders by flow	
KMT2Ar	86% (6/7)
NPM1m	67% (2/3)
Proceeded to HSCT post-revumenib	29% (4/14)
Received revumenib post-HSCT	3 pts (2 resumed post-HSCT, 1 started post-HSCT without prior rev Tx)
Patients treated for NPM1m MRD positivity (n=2)	
Achieved MRD negativity	50% (1/2)

# SAVE trial: High rates of CR & MRD negativity in newly diagnosed AML cohort treated with revumenib + venetoclax/oral HMA

ASH 2025 abstract #47

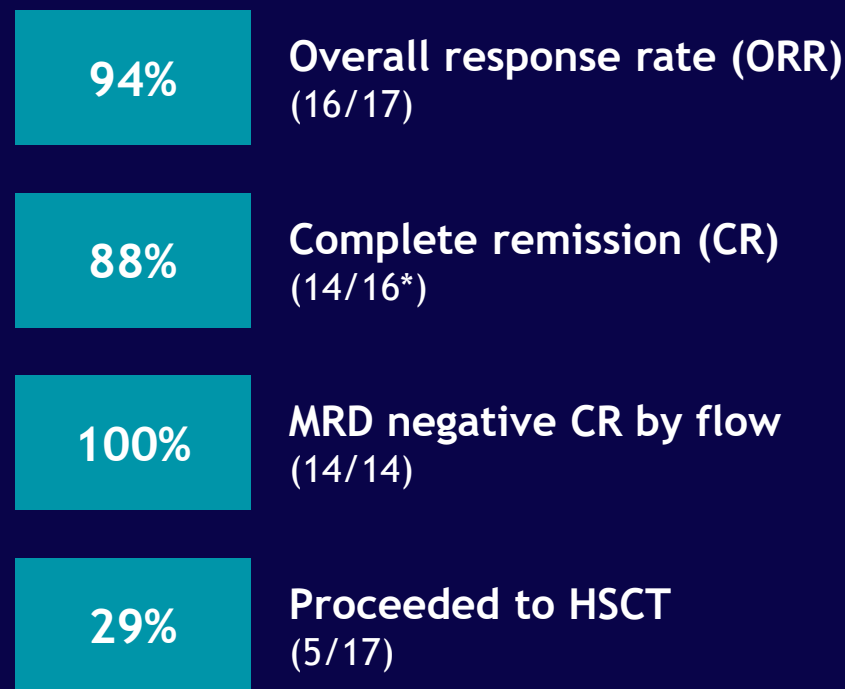
## Demographics/characteristics in ND cohort (n=17)

Age, median (range)	68 (60-83)
NPM1m/KMT2Ar	11 (65%)/ 6 (35%)
ELN22 risk	
Favorable	9 (53%)
Intermediate	2 (12%)
Adverse	6 (35%)

## Safety overview

- Most common AE was infection, occurring in 53% of pts (all G3)
- QTc prolongation in 8 (47%) pts (G1 or G2 only)
- DS in 4 (24%) pts (no events above G3)

## Efficacy in ND cohort (n=17)



At a median follow-up of 6 months, median overall survival and event free survival were not reached

# Two Phase 1 trials of revumenib with 7+3 in newly diagnosed AML show high activity, tolerability, and rapid count recovery

## Preliminary results from Ph 1b NCI study ASH Abstract #5206

### Demographics & baseline characteristics (n=12)

Age, median (range)	55 (26-72)
Genetics, n	6 high risk NPM1m, 6 KMT2Ar

### Efficacy evaluable pts at DL1 or DL2\* (n=9)

CR	89% (8/9)
CR among KMT2Ar	100% (5/5)
CR among high-risk NPM1m	75% (3/4)
Proceeded to HSCT	44% (4/9)
Time to full count recovery among pts with CR, median	25.5 days

### Safety overview

- Revumenib with 7+3 overall appears to be well-tolerated
- 9 pts completed induction DLT period: 1 DLT on DL2 (G5 typhlitis)
- 8 pts completed consolidation DLT period with no DLTs

Abstract data cutoff: July 19, 2025; Full count recovery defined as ANC >1,000 and platelets >100,000

## Preliminary results from Ph 1 Syndax study ASH Abstract #3425

### Demographics & baseline characteristics (n=7)

Age, median (range)	37 (27-56)
Genetics, n	7 KMT2Ar

### Efficacy evaluable pts at DL1\* (n=7)

CR among KMT2Ar	100% (7/7)
MRD negative CR	100% (6/6 <sup>1</sup> )
Proceeded to HSCT	57% (4/7)

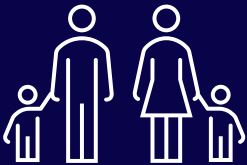
### Safety overview

- 6/7 pts. at DL1 were DLT evaluable; 1 DLT of G3 QTc prolongation (pt discontinued rev during cycle 1; pt achieved MRD(-) CR at end of cycle 1 and went to HSCT)
- Most common TEAEs were nausea and decreased neutrophils

Abstract data cutoff: June 3, 2025; 1. All patients tested for MRD negativity achieved MRD-negative CR by local assessment (n=6)

# Revumenib post-HSCT was well-tolerated with promising early efficacy in retrospective review of pediatric patients

ASH 2025 abstract #3461



At last follow-up (15.5 months median follow-up), all pts were alive with no relapses, yielding:

**100% estimated 1-year EFS**

## Demographics & baseline characteristics (n=10)

Age, median (range)	10 yrs (1.4-18 yrs)
Genetics, n (%)	8 (80%) KMT2Ar, 2 (20%) NUP98r

## Observations

Prior to HSCT	
Cycles of rev, median (range)	2 (1-4)
Rev usage, n (%)	5 (50%) combination, 5 (50%) monotherapy

Post-HSCT	
Days post-HSCT rev reinitiated, median (range)	111 (58-175)
Cycles of rev, median (range)	11 (1-25*)
*Study planned for rev post-HSCT for up to 1 yr; 1 pt continued for 2 yrs due to parental preference	

Safety overview (post-HSCT)

- Well-tolerated as post-HSCT maintenance in children; most common AE was thrombocytopenia (including two Gr 3 and one Gr 4, occurring primarily during first two cycles)

# ASH abstracts highlight the potential for long-term axatilimab use in R/R cGVHD and feasibility of combining with ruxolitinib in newly diagnosed cGVHD

## Long-term treatment duration & safety from AGAVE-201 trial of axatilimab in R/R cGVHD



- 33 pts from AGAVE-201 (N=239) were still on axa as of March 2025, with a **median of 2.8 years on therapy** (range 2.6-3.4 yrs)
- Long-term data show a continued tolerable safety profile with prolonged use

ASH 2025 abstract #6010

## Safety and feasibility of 0.6 mg/kg every 4-week axatilimab dosing in AGAVE-201 trial



- 32% (19/59) of pts who had a response on axa 0.3 mg/kg Q2W (FDA-approved dose) transitioned to 0.6 mg/kg Q4W in AGAVE-201
- Among the 19 pts who switched, the Q4W dosing was well tolerated, with a **median of 1.7 years on therapy** (range 0.2-2.7 yrs) after the dosing change



ASH 2025 abstract #272

## Interim safety analysis from Ph 2 trial of axatilimab + ruxolitinib in newly diagnosed cGVHD

- 44 pts were enrolled across 3 arms (axa+rux, rux, or corticosteroids) at the interim analysis
- **Axatilimab + ruxolitinib in newly diagnosed cGVHD patients was well tolerated** with no evidence of additive toxicity

ASH 2025 abstract #6012

# Strong financial position driven by growing Revuforj and Niktimvo contributions and stable expense outlook

Key 3Q25 Financial Results (Unaudited)		Three Months Ended Sept 30 (\$ in millions)	
		2025	2024
Product revenue, net		32.0	-
Collaboration revenue, net		13.9	-
Milestone and license revenue		-	12.5
<b>Total revenues</b>		<b>\$45.9</b>	<b>\$12.5</b>
Cost of product sales		(2.1)	-
Research & development		(56.3)	(71.0)
Selling, general and administrative		(44.9)	(31.1)
<b>Total operating expenses</b>		<b>(\$103.3)</b>	<b>(\$102.1)</b>
Other (expense) income, net		(3.3)	5.5
<b>Net loss</b>		<b>(\$60.7)</b>	<b>(\$84.1)</b>

AS OF SEPT 30, 2025:

**\$456M**  
in cash and equivalents<sup>1</sup>

**87.2M**  
shares outstanding<sup>2</sup>

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Strong balance sheet, increasing contributions from Revuforj & Niktimvo, and a stable expense outlook expected to drive **path to profitability**



Two first- &  
best-in-class drugs



**\$5B+ TAM**



**\$5B+ TAM**



Two exceptional  
product launches

Syndax is on the  
road to profitability  
with two medicines  
with multi-billion-  
dollar potential





*Lilah, diagnosed  
with R/R AML*

**FUELED BY A  
PASSION FOR  
PATIENTS**

Syndax 



