ZEVRA THERAPEUTICS

A Rare Approach
TO THERAPEUTICS

November 2026

Nasdaq: ZVRA



Adult living with Niemann-Pick disease type C

CRC-ZEV-26-0006-11

Forward Looking Statements

This presentation may contain forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements relate to future events or our future financial performance. We generally identify forward-looking statements by terminology such as “may,” “will,” “would,” “should,” “expects,” “plans,” “anticipates,” “could,” “intends,” “target,” “projects,” “contemplates,” “believes,” “estimates,” “predicts,” “assume,” “intend,” “potential,” “continue” or other similar words or the negative of these terms. We have based these forward-looking statements largely on our current expectations about future events and financial trends that we believe may affect our business, financial condition and results of operations. Forward-looking statements are not guarantees of future actions or performance. These forward-looking statements include statements regarding our anticipated financial performance, our industry, our intellectual property, business strategy, plans, goals and expectations, future operations, the timing or results of any regulatory submissions, the potential uses or benefits of MIPLYFFA, celiprolol or any other product candidates, the success or timing of the launch or commercialization of any products, and our strategic and product development objectives. These forward-looking statements are based on information currently available to Zevra and its current plans or expectations and are subject to a number of known and unknown uncertainties, risks and other important factors - including those discussed under the caption “Risk Factors” in Zevra’s Annual Report on Form 10-K for the year ended December 31, 2025, filed on March 9, 2026, and in our other filings with the SEC – and could cause actual results, performance, or achievements to differ materially from those indicated by the forward-looking statements made herein. While we may elect to update such forward-looking statements at some point in the future, except as required by law, we disclaim any obligation to do so, even if subsequent events cause our views to change. Although we believe the expectations reflected in such forward-looking statements are reasonable, we can give no assurance that such expectations will prove to be correct. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to this presentation.

This presentation also may contain estimates and other statistical data made by independent parties and by us relating to market size and other data about our industry. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. In addition, projections, assumptions and estimates of our future performance and the future performance of the markets in which we operate are necessarily subject to a high degree of uncertainty and risk.

We are a Purpose Driven Company

OUR MISSION

We redefine what is possible
in bringing life-changing therapies to people living with rare diseases

OUR VISION

To become a leading rare disease therapeutics company
that is driven by patient insights and innovation to make a transformational impact on the people we serve

OUR VALUES



Patient Centricity
prioritizing patient needs



Accountability
delivering on commitments consistently



Integrity
always doing what is right



Innovation
transforming possibilities into reality



Courage
acting with strength and conviction

Unique Opportunity to Impact People Living with Rare Diseases

- **Commercial-stage** rare disease products
- **Late-stage** clinical development assets
- **MAA under review by EMA**
- **Existing infrastructure** can be leveraged for future growth

Multiple revenue sources generated strong year-to-date net revenue of **$113.6M**[i]

Exited Q3 26 with a **strong cash position**: **$270.9M**[ii]

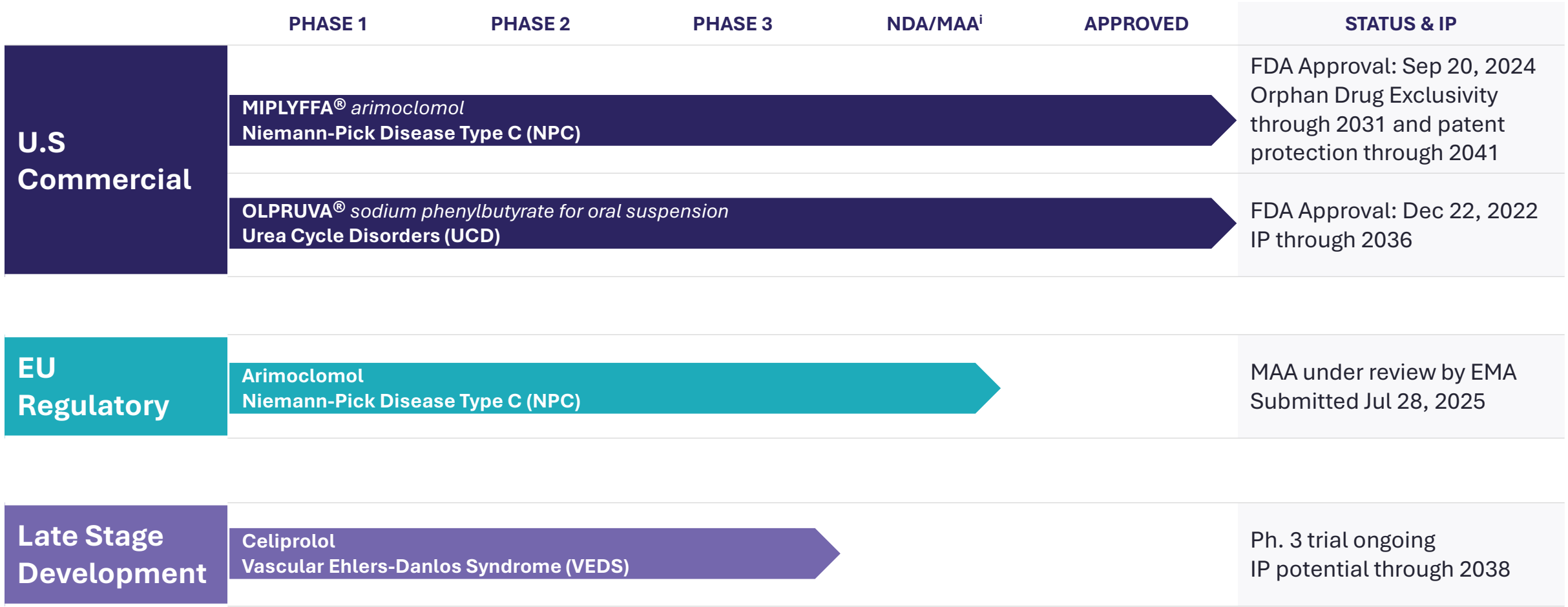
Growth mode; operating runway to execute



Adult living with NPC

i. As of September 30, 2026; ii. Cash, cash equivalents and investments as of September 30, 2026

Diversified Portfolio Delivering Value for Patients



Certain products may be subject to royalty obligations, details and required disclosures are available in our SEC filings or on our website: www.zevra.com.

i. New Drug Application\Marketing Authorization Application;

The safety and efficacy of product candidates have not been established. There is no guarantee that these products will receive health authority approval or become commercially available for the uses being investigated.

Fulfilling Our Mission to Bring Life-Changing Therapies to People Living with Rare Disease

Focus | **Execute** | **Innovate**

Resources Prioritized Across Four Pillars to Achieve our Vision

Marketed Therapies

Growth mode for MIPLYFFA® to deliver meaningful benefit to patients



Pipeline and Innovation

Synergistic growth opportunities



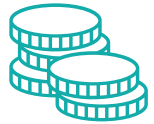
Talent and Culture

Experienced team with rare disease expertise



Corporate Foundation

Strong balance sheet with financial discipline



Executing Against Key Growth Drivers in 2026

Marketed Products

Established MIPLYFFA as Foundational Treatment for NPC



- Adoption achieved in 50%+ of estimated diagnosed patients[1]
- Pediatric sNDA filing anticipated in Q1 2027[i]

Pipeline and Innovation

Significant Growth Opportunities



- Arimoclomol MAA under review by EMA
- 134 patients in global expanded access program
- Pivotal Phase 3 trial underway for celiprolol; engaged FDA in Type C meeting to discuss regulatory options

Talent and Culture

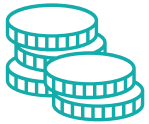
Becoming "Partner of Choice" for Rare Disease Products



- Demonstrating leadership through advocacy, community engagement, and patient and caregiver support services
- Established presence in rare disease biotech hub

Corporate Foundation

Strengthened Capital Position Through $50M SDX Portfolio Sale



- Debt-free balance sheet
- Demonstrated operational discipline
- Bolstered financial flexibility

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i. Supplemental New Drug Application (sNDA)

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Marketed Therapies



Adult living with NPC

Niemann-Pick disease type C (NPC) is an ultra-rare genetic disorder that leads to premature mortality.

For full prescribing information, visit MIPLYFFA.com

NPC is a Devastating and Fatal Lysosomal Storage Disorder

~900 people in the U.S. with NPC, 300–350 diagnosed or treated[1]

Cholesterol Buildup Leads to Cell Death



NPC gene mutations produce abnormal, absent, or non-functional NPC proteins[2]

Progressive lipid build up leads to cell death and ultimately organ dysfunction in the spleen, liver, and brain

Heterogenous Presentation



Age of Onset

Split 50/50 (children/adults)

Rate of Progression

Presentation of Symptoms

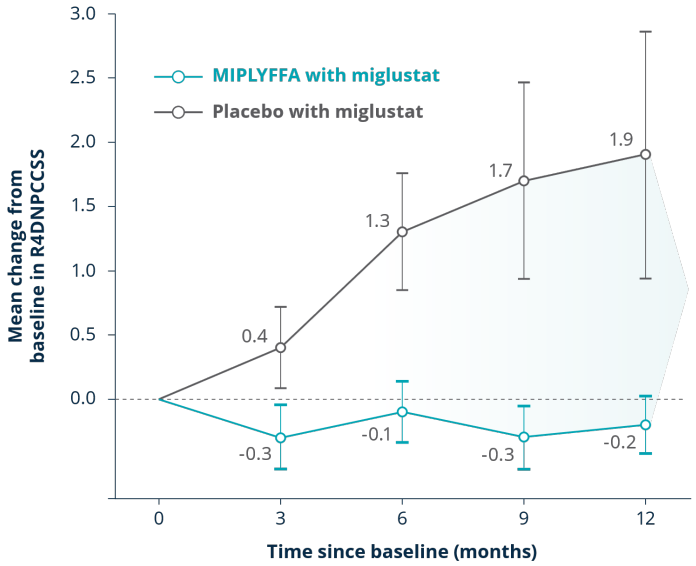
Signs and Symptoms of NPC:



Cognition	Speech	Ambulation	Swallow	Fine Motor Skills	Visceral

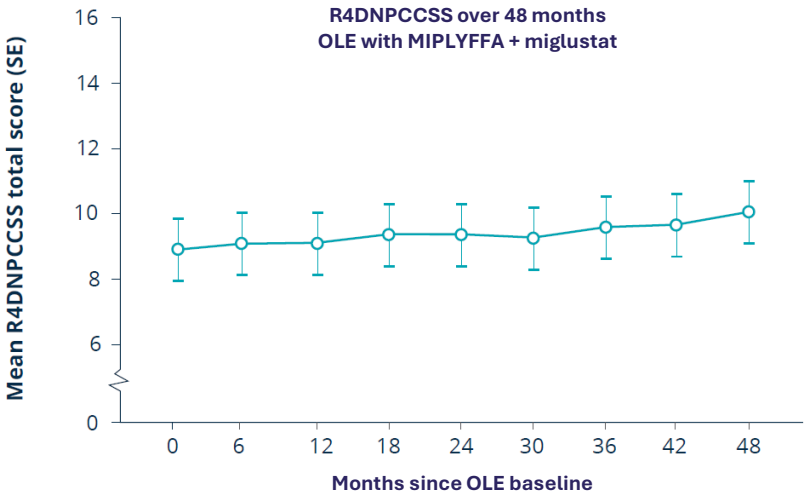
Multiple Trials Demonstrate MIPLYFFA's Impact on NPC Progression

Proven effectiveness using the NPC Clinical Severity Scale in combination with miglustat [i,3, 4]



MIPLYFFA®
arimoclomol capsules

~80%
Of patients who participated in the Phase 2/3 clinical trial took miglustat[3]



Changing the Therapeutic Landscape of NPC

Improvement
at first evaluation Week 12[3,4]

Halts Disease Progression
at 12 months[3,4]

Durable effect for
5+ Years[3,4]

[i]Measured by the R4DNPCCSS score: ambulation, speech, swallow, and fine motor skills. 80% of patients were taking miglustat.

For full prescribing information, visit MIPLYFFA.com

Establishing MIPLYFFA as Foundational Treatment for NPC

Demonstrating Clinical Differentiation	Driving Patient Access	Increasing Identification of Patients
• Unique **mechanism of action** improves cholesterol trafficking through improved lysosomal function and upregulation of genes belonging to the CLEAR network[i,5] • Strength of **clinical data** • Magnitude of **safety database** • Robust and expanding **body of evidence**	• Payor coverage at **69%** of covered lives[ii] • Patients gaining coverage through **Medical Exception Pathways,** if not on formulary • Driving conversion of Patient Enrollments and High Adherence Rates	• **197** patient enrollments[ii] • Implemented **genetic testing** collaborations and **disease awareness** campaigns to identify newly diagnosed patients • Leveraging **AI predictive model** of claims data to find potential patients

MIPLYFFA is the first FDA-approved treatment for NPC, supported by a growing body of real-world evidence.

i. Coordinated Lysosomal Expression and Regulation (CLEAR); ii. As of Q3 26

Driving the Next Wave of Growth for MIPLYFFA

U.S. Prevalence

~900 individuals in the U.S. live with NPC of which 300-350 are diagnosed or treated

- Continuing to drive penetration amongst currently Dx patients
- Finding Dx but not treated patients
- Helping get new patients Dx & treated earlier

EU Prevalence

~1,100 individuals living with NPC in Europe[5]
Diagnosis rates **estimated to be higher** than the U.S.

- MAA under review
- Submission includes most expansive data set available in NPC

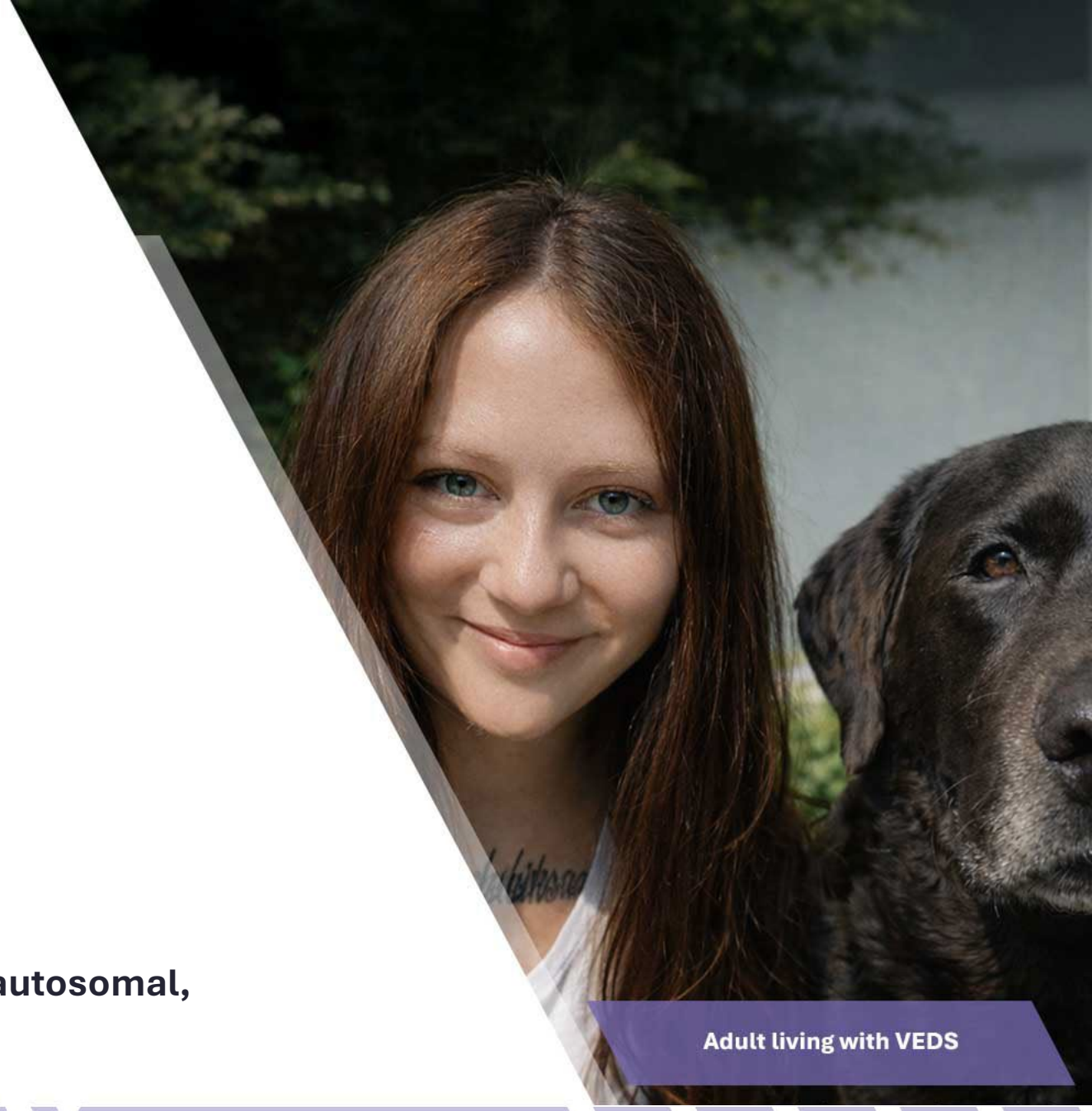
Expanded Access

Geographic expansion to broaden access beyond the U.S. and Europe

- Expanding EAP to support more patients
- Supporting a greater number of patients
- Furthers mission to provide access

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Pipeline and Innovation



Vascular Ehlers-Danlos Syndrome (VEDS) is a severe autosomal, dominant, genetic, connective tissue disorder.

VEDS is a Life-threatening Connective Tissue Disorder with No Approved Treatments

VEDS is the most severe Ehlers-Danlos syndrome subtype[6]

Inherited **connective tissue disorder** caused by ***COL3A1*** gene mutations

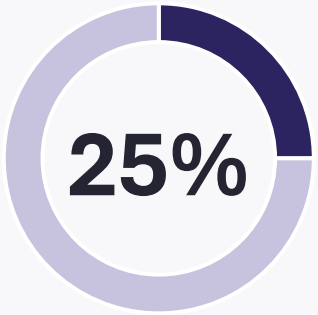
Leads to defect in type III procollagen in vessel walls and hollow organs

Characterized by **arterial aneurysms** and **hollow organ ruptures**[6]

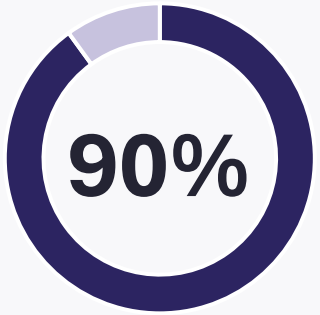
~7,500 individuals in the U.S. diagnosed and live with VEDS[7]

95% of diagnoses are genetically confirmed (*COL3A1*)[7, 8]





~25% of patients experience an event **before the age of 20**[7]



~90% of patients experience an event **by the age of 40**[7]

Median survival age

51 YEARS

Arterial rupture being the **most common** cause of sudden death[7]

Celiprolol is a Potential Treatment of Patients with *COL3A1*+ VEDS

- Celiprolol addresses VEDS by reducing mechanical stress on collagen fibers within arterial wall[9]
- New chemical entity in the U.S., Orphan drug & breakthrough therapy designation
- Selective adrenergic modulator (SAM)

Consistent Reduction in Major Vascular Events Across Previously Completed Studies [10,11,12]

- **Randomized & real-world data across BBEST and two long-term European cohorts**
- **~5% annual major vascular event rate on celiprolol vs ~12% untreated**
- **Improved survival observed in treated patients**

DiSCOVER TRIAL Phase 3 DiSCOVER Trial Ongoing

- **Decentralized pivotal trial (in-home & virtual)**
- **Special Protocol Assessment (SPA)**
- **Designed to confirm clinical benefit seen in prior studies**
- **72 of 150 patients enrolled as of Q3 26**
- **Interim analysis at 28 events; final at 46 events**

Celiprolol is the primary treatment for VEDS patients in several European countries[6]

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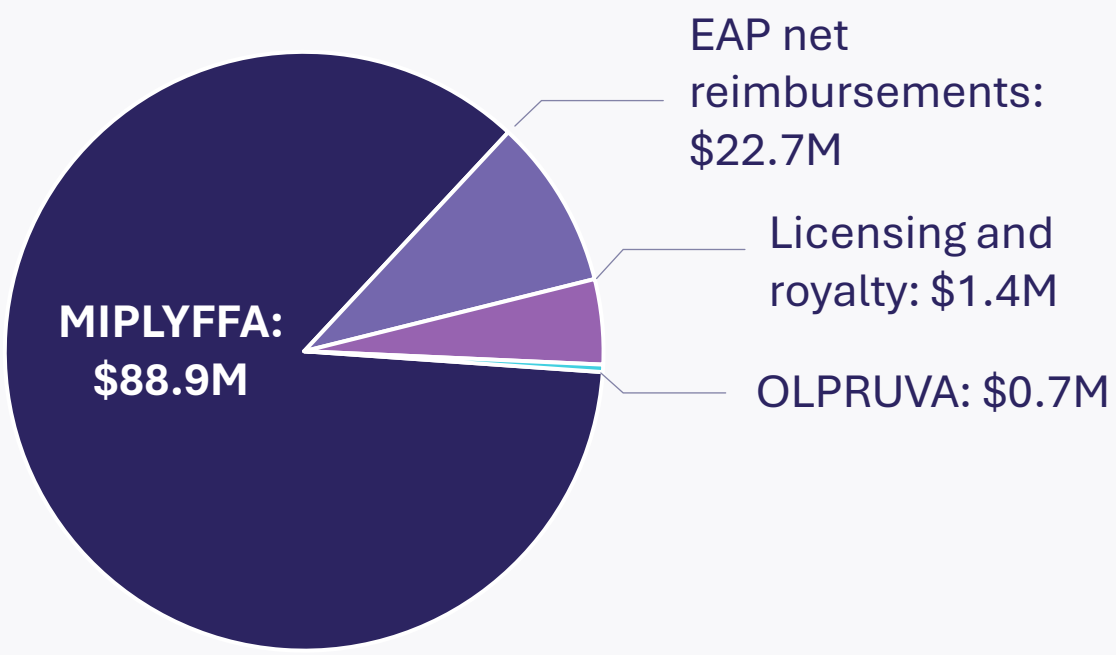
Strong Corporate Foundation

Demonstrating Financial Discipline

Financial Position is a Source of Strength

2026 Income Statement Details[i]

Net revenue of $ 113.6M[i]



- Q3 MIPLYFFA US revenue of $34.0 million, a 12.6% increase over Q2
- 2026 net income of $65.9M, or $1.07 per basic share and $1.03 per diluted share, which includes non-cash fair value adjustment charge of $0.3M, non-cash stock-based compensation expense of $8.7M, and non-cash intangible asset amortization of $0.9M[i]

Balance Sheet as of September 30, 2026:

- **Available cash, cash equivalents and investments of $270.9M and no debt**

i. 2026 financial results through September 30, 2026

Growth Drivers to Maximize the Opportunity in 2026 and Beyond

Commercial Execution to Further Unlock NPC Opportunity in the U.S.



- Continue **execution of U.S. launch** to drive access to prevalent patient population
- Leveraging **genetic testing** data to help identify undiagnosed patients
- **Publication strategy** to drive clinical differentiation and continuing education

Strengthened IP protection for MIPLYFFA



- MIPLYFFA patent submitted for Orange Book listing, with **patent protection to 2041**
- **Additional IP filed** with USPTO in 2024 and 2025 to further **enhance exclusivity** and support global growth

MAA Submitted for Arimoclomol



- **Continue to drive EU experience** and KOL network through EAP
- Execute **go to market strategy** in Europe



Geographic expansion to broaden access to patients beyond U.S. and Europe

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A Rare Approach to Therapeutics

Nasdaq: ZVRA



Children living with NPC

Sources

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