



A Rare Approach TO THERAPEUTICS

September 2026

Nasdaq: ZVRA



Adult living with Niemann-Pick
disease type C

CRC-ZEV-26-0006-10

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 includes references to certain non-GAAP financial measures, including adjusted net income and adjusted net income per share. Quantitative reconciliations of these measures to the most directly comparable GAAP measures, as well as information regarding the usefulness of these measures to management and investors, are included in our earnings press release issued today, which is available on our website.

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
H1 2026 net revenue
of **\$75.9M**

Exited Q2 26
with a **strong
cash position:**
\$260.2Mⁱ

**Growth mode;
operating runway
to execute**



Adult living with NPC

Diversified Portfolio Delivering Value for Patients

	PHASE 1	PHASE 2	PHASE 3	NDA/MAA ⁱ	APPROVED	STATUS & IP
U.S Commercial	MIPLYFFA[®] <i>arimoclomol</i> Niemann-Pick Disease Type C (NPC)					FDA Approval: Sep 20, 2024 Orphan Drug Exclusivity through 2031 and patent protection through 2041
	OLPRUVA[®] <i>sodium phenylbutyrate for oral suspension</i> Urea Cycle Disorders (UCD)					FDA Approval: Dec 22, 2022 IP through 2036
EU Regulatory	Arimoclomol Niemann-Pick Disease Type C (NPC)					MAA under review by EMA Submitted Jul 28, 2025
Late Stage Development	Celiprolol Vascular Ehlers-Danlos Syndrome (VEDS)					Ph. 3 trial ongoing IP potential through 2038

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 diagnosed patients

Pipeline and Innovation

**Significant
Growth
Opportunities**



- Arimoclomol MAA under review by EMA
- 132 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



Marketed Therapies

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

For full prescribing information, visit [MIPLYFFA.com](https://www.miplifyffa.com)



Adult living with NPC

NPC is a Devastating and Fatal Lysosomal Storage Disorder

~900 people in the U.S. with NPC, 300–350 diagnosed or treated¹

Cholesterol Buildup Leads to Cell Death



NPC gene mutations produce abnormal, absent, or non-functional NPC proteins²

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



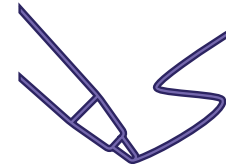
Speech



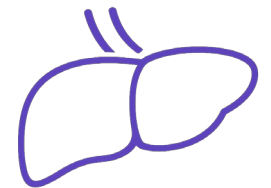
Ambulation



Swallow



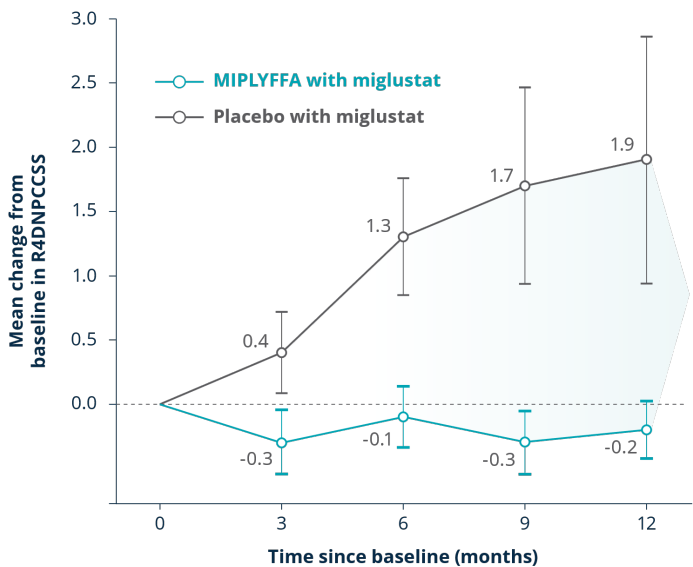
Fine Motor Skills



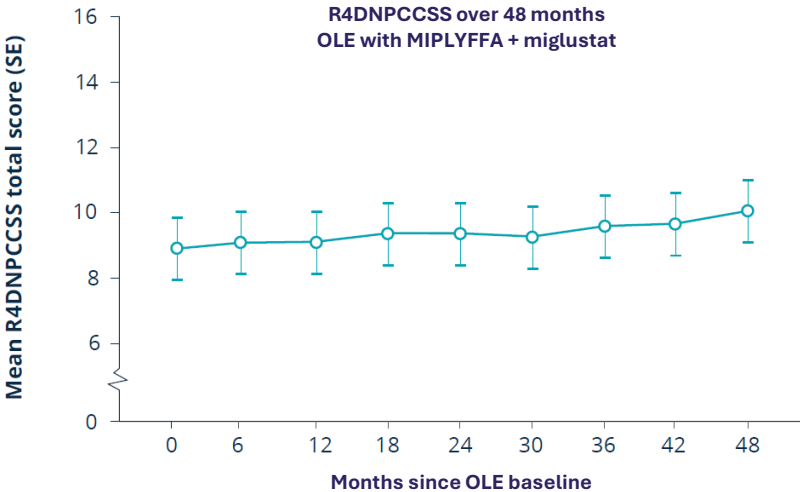
Visceral

Multiple Trials Demonstrate MIPLYFFA's Impact on NPC Progression

Proven effectiveness using the NPC Clinical Severity Scale in combination with miglustat ^{i,3, 4}



~80%
Of patients who participated in the Phase 2/3 clinical trial took miglustat³



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}

ⁱMeasured by the R4DNPCSS score: ambulation, speech, swallow, and fine motor skills. 80% of patients were taking miglustat.

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 livesⁱⁱ • Patients gaining coverage through Medical Exception Pathways, if not on formulary • Driving conversion of Patient Enrollments and High Adherence Rates	• 184 patient enrollmentsⁱⁱ • Programs implemented to find undiagnosed patients yielding encouraging results • Expanded genetic Dx collaboration yielding new patient identification • Initiated AI predictive model to find potential patients

Independent market research suggests MIPLYFFA is the preferred NPC therapy most trusted by clinicians and shown to improve balance, swallowing, cognition, speech, and reduce falls.

Capturing 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 Europe⁵
Diagnosis rates **estimated to be higher** than the U.S.

- MAA under review by EMA
- Submission includes most expansive data set available in NPC
- Expanding EAP to support more patients

Expanded Access

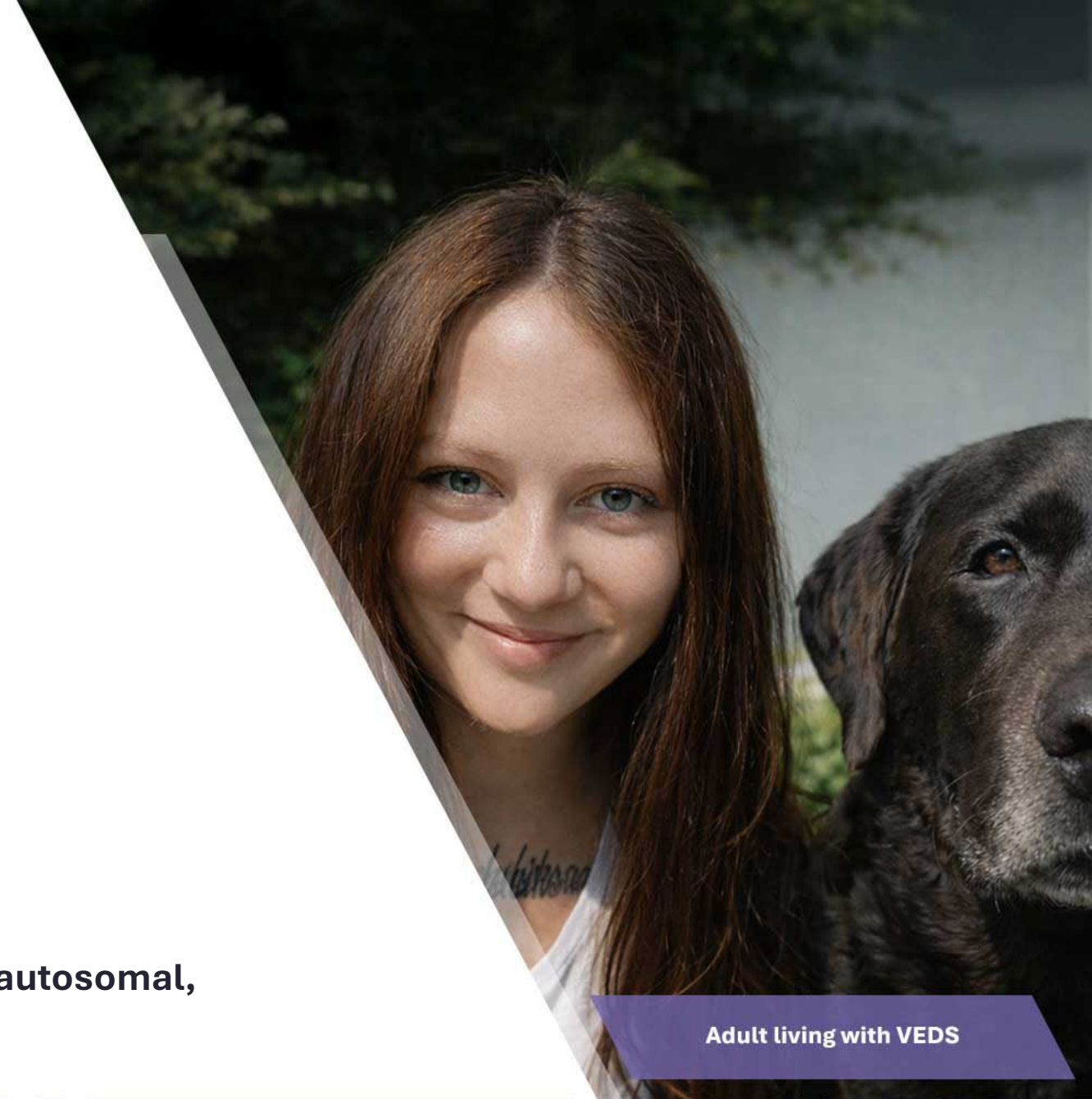
Geographic expansion to broaden access outside the U.S. and Europe

- Supporting a greater number of patients
- Furthers mission to provide access
- Building distributor network in select territories



Pipeline and Innovation

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

A photograph of a young woman with long brown hair and blue eyes, smiling. She has a tattoo on her neck. A black dog is partially visible next to her. The photo is set against a background of green foliage.

Adult living with VEDS

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

VEDS is the **most severe** Ehlers-Danlos syndrome subtype⁶



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



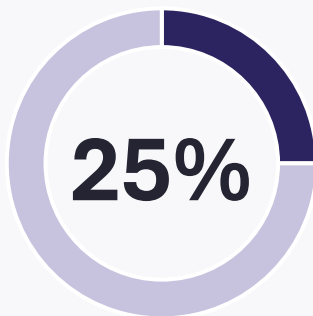
Leads to **defect** in type III procollagen in vessel walls and hollow organs



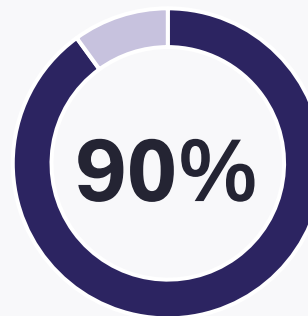
Characterized by **arterial aneurysms** and **hollow organ ruptures**⁶

~7,500 individuals in the U.S. diagnosed and live with VEDS⁷

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 death⁷

Celiprolol is a Potential Treatment of Patients with COL3A1+ VEDS

- Celiprolol addresses VEDS by reducing mechanical stress on collagen fibers within arterial wall⁹
- 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
- 66 of 150 patients enrolled as of Q2 26
- Interim analysis at 28 events; final at 46 events

Celiprolol is the primary treatment for VEDS patients in several European countries⁶



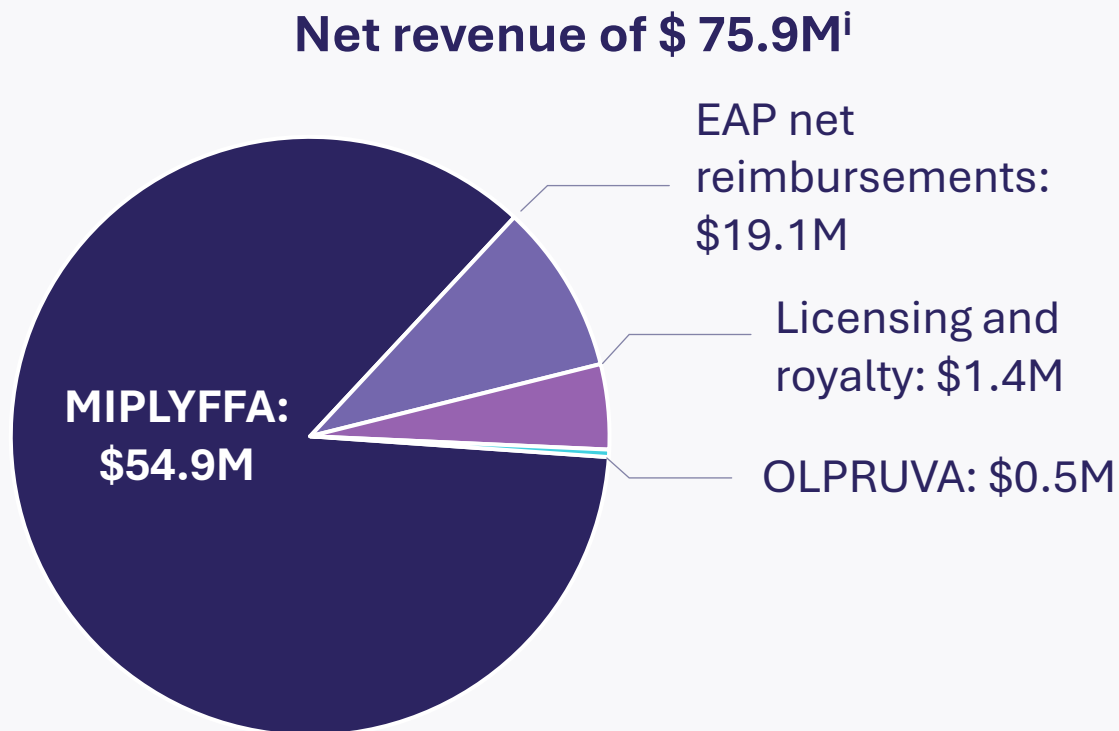
Strong Corporate Foundation

Demonstrating Financial Discipline



Financial Position is a Source of Strength

2026 H1 Income Statement Detailsⁱ



- Q2 MIPLYFFA US revenue of \$30.2 million, a 23% increase over Q1
- H1 2026 net income of \$46.6M, or \$0.76 per basic share and \$0.74 per diluted share, which includes non-cash fair value adjustment charge of \$5.4M, non-cash stock-based compensation expense of \$6.1M, and non-cash intangible asset amortization of \$0.6M

Balance Sheet as of June 30, 2026:

- Available cash, cash equivalents and investments of **\$260.2M** and no debt

Growth Drivers to Maximize the Opportunity in 2026 and Beyond

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



- Continue **successful execution of U.S. launch** to drive access to prevalent patient population
- **Genetic testing** collaborations to find 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; Potential for EMA Approval of Arimoclomol



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



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



A Rare Approach to Therapeutics

Nasdaq: ZVRA



Children living with NPC

Sources

1. Burton et.al., Molecular Genetics and Metabolism Volume 134, Issues 1–2, September–October 2021, Pages 182-187
2. Geberhiwot T, et al. Orphanet J Rare Dis. 2018 Apr 6;13(1):50.
3. MIPLYFFA Full Prescribing Information. Celebration, FL, US, Zevra Therapeutics Inc.
4. Mengel et al., Molecular Genetics and Metabolism Volume 145, Issue 4, August 2025, 109189. <https://doi.org/10.1016/j.ymgme.2025.109189>
5. Patterson M. Niemann-Pick Disease Type C. 2000 Jan 26 [Updated 2020 Dec 10]. In: Adam MP, Feldman J, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2025. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK1296/>
6. FightvEds.org
7. Pepin M, et al. Genet Med. 2014 Dec;16(12):881-8
8. Leistritz, D., Pepin, M., Schwarze, U. et al. COL3A1 haploinsufficiency results in a variety of Ehlers-Danlos syndrome type IV with delayed onset of complications and longer life expectancy. Genet Med 13, 717–722 (2011). <https://doi.org/10.1097/GIM.0b013e3182180c89>
9. Nawarskas J, et al. *Cardiology in Review*: Celiprolol: a Unique Selective Adrenoceptor Modulator. Sept/Oct 2017, Volume 25, Number 5 247-253
10. Ong et al., Lancet 2010; 376: 1476-1484
11. Frank et al., J Am Coll Cardiol. 2019 Apr 23;73(15):1948-1957
12. Baderkhan et al., Eur J Vasc Endovasc Surg (2021) 61, 326-331