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# **RARE** PATIENTS
# **RARE** SOLUTIONS

Corporate Presentation

July 2024

**Ella**
Living with ENPP1 Deficiency

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The words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “might,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “will,” “would” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. We may not actually achieve the plans, intentions or expectations disclosed in these forward-looking statements, and you should not place undue reliance on these forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements. For a discussion of risks and uncertainties, and other important factors, any of which could cause our actual results to differ from those contained in the forward-looking statements, see the “Risk Factors” section in our most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission, as well as discussions of potential risks, uncertainties and other important factors, in the Company’s most recent filings with the Securities and Exchange Commission.

In addition, the forward-looking statements included in this presentation represent our views as of the date of this presentation. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements in the future, we specifically disclaim any obligation to do so. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to the date of this presentation.

# Inozyme is at the forefront of developing novel therapies for rare diseases that affect bone health and blood vessel function

**ENPP1 Deficiency, ABCC6 Deficiency and calciphylaxis are serious diseases affecting bone health and blood vessel function linked to dysregulation of the PPi-Adenosine Pathway with no approved therapies**

**Lead product candidate INZ-701 demonstrated a rapid, significant, and sustained increase in PPi levels, preliminary evidence of efficacy, and a favorable safety profile across multiple clinical trials**

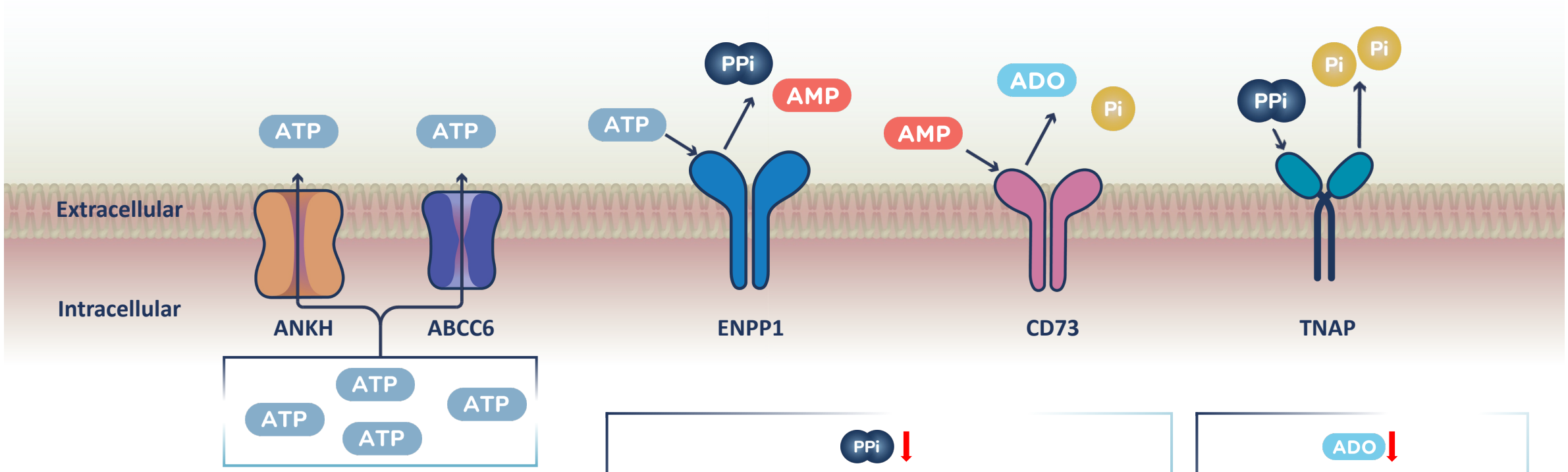
**Currently in pivotal trial for ENPP1 Deficiency; Completed Phase 2 trial for ABCC6 Deficiency and treatment period in Phase 1b trial in calciphylaxis program**

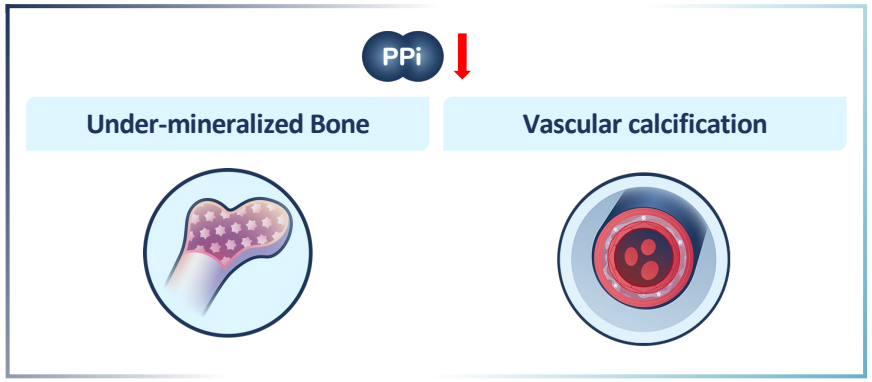
**Experienced team with a track record of success in rare disease**

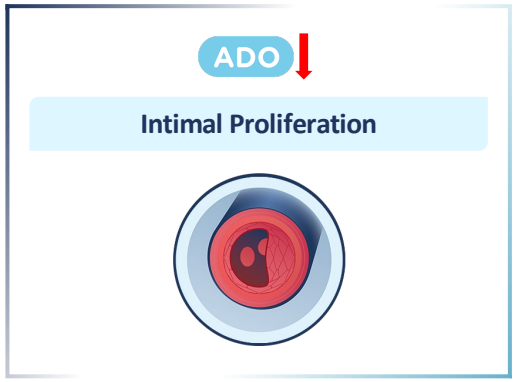
**In a position of financial strength, with several expected upcoming milestones and a pipeline designed for long-term value creation**

- $166.2M expected to fund operations into Q4 2025 as of 3/31/24
- 61.85M common shares outstanding as of 5/2/24

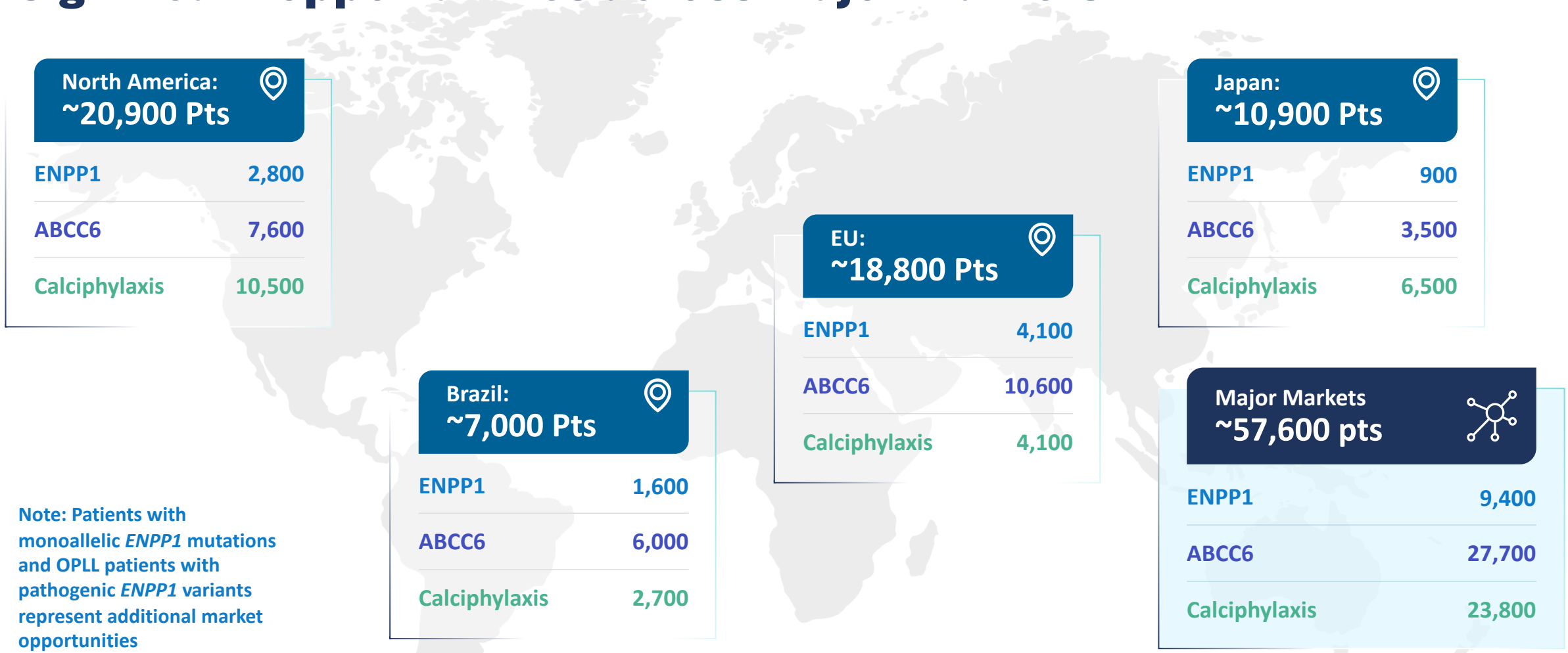
# The PPi-Adenosine Pathway regulates bone health and blood vessel function – ENPP1 plays a central role



# Diseases associated with the PPi-Adenosine Pathway present significant opportunities across major markets



**North America: ~20,900 Pts**

| | |
|---|---|
| ENPP1 | 2,800 |
| ABCC6 | 7,600 |
| Calciphylaxis | 10,500 |

**Brazil: ~7,000 Pts**

| | |
|---|---|
| ENPP1 | 1,600 |
| ABCC6 | 6,000 |
| Calciphylaxis | 2,700 |

**EU: ~18,800 Pts**

| | |
|---|---|
| ENPP1 | 4,100 |
| ABCC6 | 10,600 |
| Calciphylaxis | 4,100 |

**Japan: ~10,900 Pts**

| | |
|---|---|
| ENPP1 | 900 |
| ABCC6 | 3,500 |
| Calciphylaxis | 6,500 |

**Major Markets ~57,600 pts**

| | |
|---|---|
| ENPP1 | 9,400 |
| ABCC6 | 27,700 |
| Calciphylaxis | 23,800 |

**Note: Patients with monoallelic *ENPP1* mutations and OPLL patients with pathogenic *ENPP1* variants represent additional market opportunities**

Sources: Company estimates. Ferreira et al. Genet Med, 2021. Ferreira et al. Orphanet Journal of Rare Diseases, 2022. Nigwekar SU, et al. J Gen Intern Med. 2014; Nigwekar SU, et al. J Am Soc Nephrol. 2016. Chinnadurai, R., Huckle, A., Hegarty, J. et al. Calciphylaxis in end-stage kidney disease: outcome data from the United Kingdom Calciphylaxis Study. J Nephrol 34, 1537–1545 (2021). https://doi.org/10.1007/s40620-020-00908-9 USRDS Annual Data Report 2021. https://adr.usrds.org/2021/end-stage-renal-disease/1-incidence-prevalence-patient-characteristics-and-treatment-modalities. Supplemented ERA-EDTA Registry data evaluated the frequency of dialysis, kidney transplantation, and comprehensive conservative management for patients with kidney failure in Europe - Kidney International (kidney-international.org) Prevalence of calciphylaxis estimated to be 2% of hemodialysis patients; North America (excl Mexico); EU = EU5 + UK.

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# ENPP1 Deficiency

**Ella**
Living with ENPP1 Deficiency

# ENPP1 Deficiency is a lifelong, multisystem, rare genetic disease with high mortality and morbidity

**GACI/IIAC**
**0-1 Years (~1-2%)***

***50% mortality within 6 months of birth***

- Severe cardiovascular complications

**ARHR2 (Rickets)**
**1 to <13 years (~25-30%)***

***Impaired growth Orthopedic surgery***

- Skeletal defects: Rickets
- Hearing loss

**ARHR2 (Osteomalacia)**
**13+ Years (~65-70%)***

***Bone & joint pathology***

- Skeletal defects: Osteomalacia
- Joint, tendon, and ligament complications
- Hearing loss

| Biallelic Genetic Prevalence[1]: | 1:64,000 |
|---|---|

| | | | |
|---|---|---|---|
| PATIENTS IN US/CANADA | ~ 2,800 | PATIENTS IN JAPAN | ~ 900 |
| PATIENTS IN EUROPE | ~ 4,100 | PATIENTS IN BRAZIL | ~ 1,600 |

Note: Estimates do not include symptomatic patients with monoallelic mutations

*Estimated percent of total prevalence., 1. Ferreira et al. Orphanet Journal of Rare Diseases, 2022. GACI: Generalized Arterial Calcification of Infancy, IIAC : Idiopathic Infantile Arterial Calcification, ARHR2: Autosomal Recessive Hypophosphatemic Rickets Type 2

# Completed Phase 1/2 trial of INZ-701 in adults with ENPP1 Deficiency successfully met all study objectives

## Safety

- **Favorable safety profile** was maintained
- Low/moderate, sometimes transient, ADA titers

## PK/PD

- **PK data** from cohort 4 **support once-weekly dosing**
- **PPi remained elevated** with long-term treatment

## Clinical

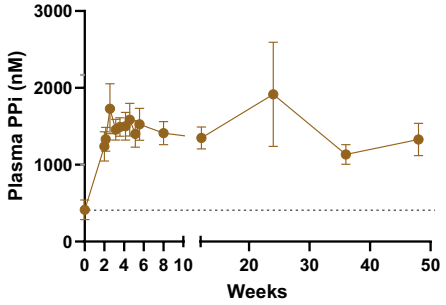
- Favorable response on **clinical outcomes** (PROs and 6MWT) was **maintained**
- Bone biomarker response consistent with restoring proper bone mineralization

# Significant increase in PPi levels were associated with improvement in phosphate and FGF-23 and supports MOA

## Pooled Cohorts 1-3: Baseline vs mean Week 2-48 PPi, FGF-23, and Pi levels (±SEM)

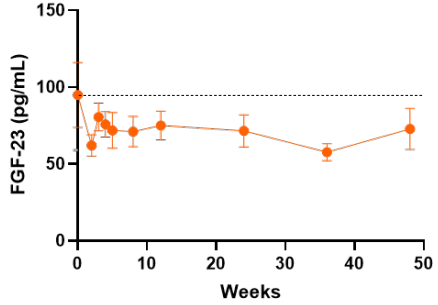
**Plasma PPi**

Ref range : 1002-2169nM



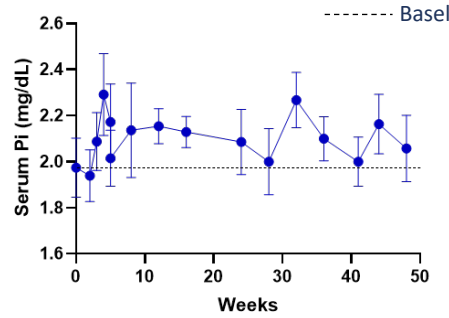

**Serum FGF-23**

Ref range : <59pg/mL



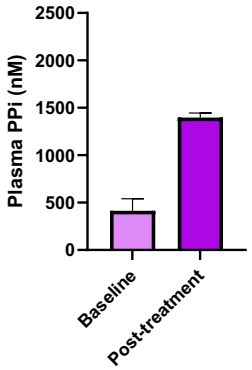

**Serum Pi**

Ref range : 2.5-4.5mg/dL

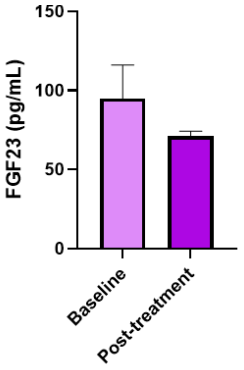



## Pooled Cohorts 1-3: Mean PPi, FGF-23 and Pi levels (±SEM)

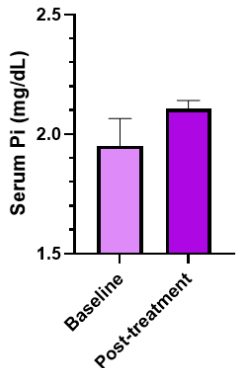
**Plasma PPi**




**Serum FGF-23**




**Serum Pi**




Note: Serum Pi increases observed in absence of phosphate and active vitamin D supplementation

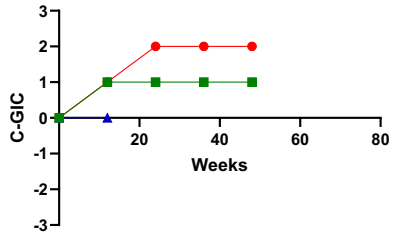
Data cut Jan 25 2024 ; Data presented as mean ± SEM

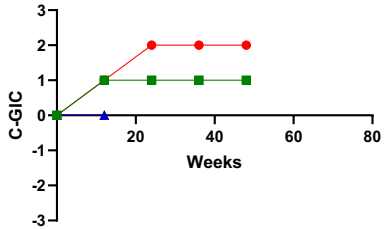
# Global Impression of Change Scale: Concordant improvement in C-GIC and P-GIC in all three dose cohorts

Clinician's Global Impression

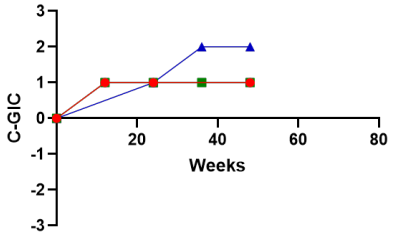
Patient's Global Impression

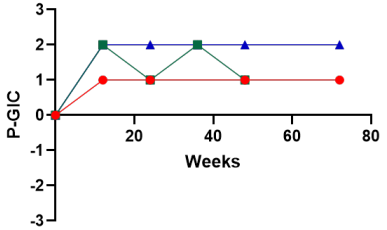
## Cohort 1 (0.2mg/kg) (n=3)



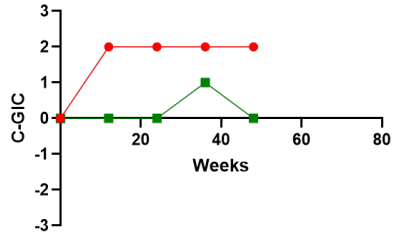



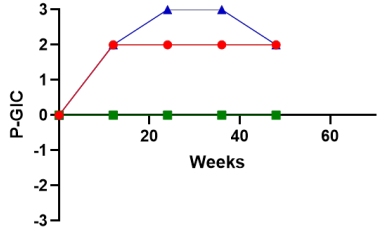

## Cohort 2 (0.6mg/kg) (n=3)




## Cohort 3 (1.8mg/kg) (n=3)*




| -3 | -2 | -1 | 0 | 1 | 2 | 3 |
|---|---|---|---|---|---|---|
| Very much worse | Much worse | Minimally worse | No Change | Minimally improved | Much improved | Very Much improved |

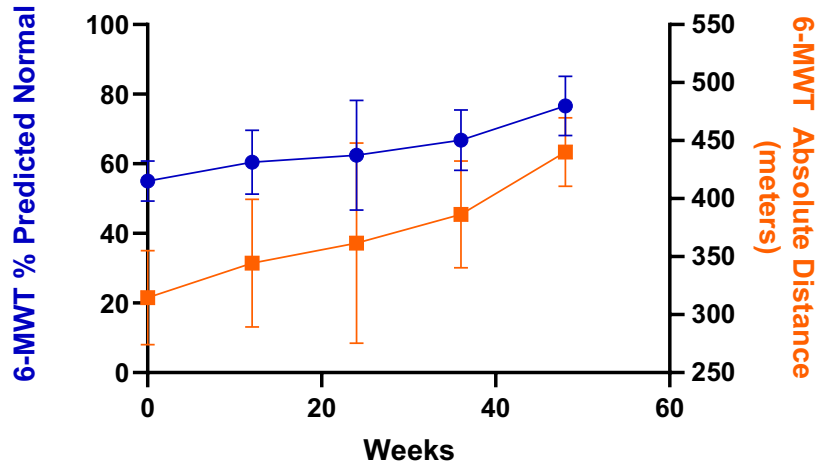
* n=2 for C-GIC

Data cut 25 Jan 24

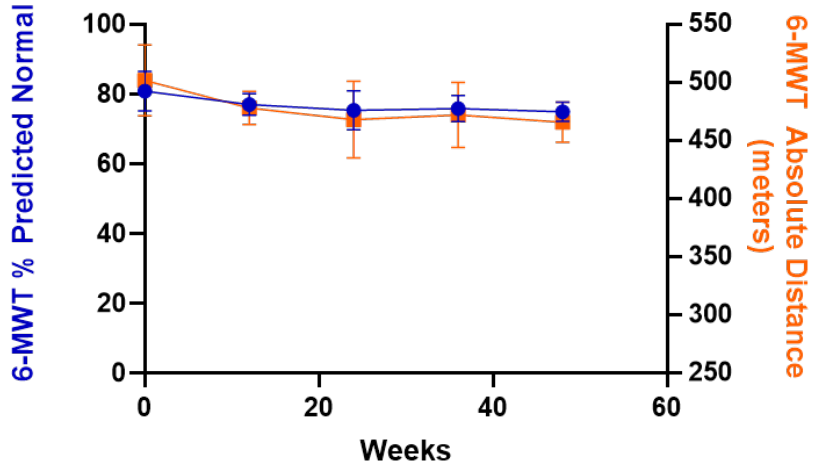
Colors represent individual patients in respective cohorts

# INZ-701 showed trend for improvement in 6-minute walk test (6-MWT)

**Patients with <70% predicted of healthy 6-MWT at baseline (n=5)**




**Patients with >70% predicted of healthy 6-MWT at baseline (n=4)**




- Greater improvement observed in patients with poor baseline 6-MWT
- Stable 6-MWT scores observed in patients with higher baseline values

Data cut Jan 25, 2024; Data presented as ± SEM

# ENPP1 Deficiency: Planned Path to Global Approval

Pediatric Trial in Pediatric ENPP1 Deficiency Ongoing

# ENERGY-3: Pivotal trial in pediatric patients with ENPP1 Deficiency (ARHR2)

## Patient recruitment underway – Topline data expected mid-2025

**Population: Pediatric**

- Confirmed genetic diagnosis
- Radiographic evidence of skeletal abnormalities
- ≥1 year and <13 years
- Low plasma PPi

**Design: Randomized (2:1), Open Label**

| | Randomized Treatment Period | Open Label Extension Period |
|---|---|---|
| n = up to 33 | Randomization: INZ-701 Arm – 2.4 mg/kg weekly; Control Arm – Conventional Therapy; 52 weeks | All patients to receive INZ-701 beyond 52 weeks |

Multicenter, Multinational

**Endpoints**

**US**

- **Primary**: Change in plasma PPi from baseline over time
- **Secondary**: Trends in RGI-C score, RSS, Growth Z-score; PK

**EU**

- **Co-Primary**:
  - Change in plasma PPi from baseline over time
  - RGI-C score (with p<0.2)
- **Secondary**: RSS, Growth Z-score; PK

RGI-C: Radiographic Global Impression of Change, RSS: Rickets Severity Score, PK: Pharmacokinetic, ARHR2: Autosomal Recessive Hypophosphatemic Rickets Type 2, ClinicalTrials.gov: NCT06046820

# Planned path to global approval of INZ-701 in ENPP1 Deficiency

| | Endpoints |
|---|---|
| **ENERGY-1: Infant** (0-12 mos.)<br>Phase 1b<br>Single arm (n=8) | ***Safety*** *and* ***tolerability*** *as primary;* ***PPi*** *and* ***survival*** *as secondary* |
| **ENERGY-2: Infant** (0-12 mos.)<br>Pivotal<br>Single arm per agreed PIP**<br>(n=12) | ***PPi + survival*** *as co-primary* |
| **ENERGY-3: Pediatric**<br>**(**≥1-<13 yrs.)<br>Pivotal<br>Randomized – 2:1 (n=33) | ***PPi*** *as sole primary* (US) and co-primary with* ***RGI-C*** *(EU)* |
| **ENERGY-4: Adolescent and Adult** (13+)<br>Pivotal<br>Randomized – 2:1 (n=30)*** | ***PPi*** *as sole primary* (US) and co-primary with* ***BMC/BMD*** *expected (EU)* |

## Basis for Planned Marketing Applications

### 1st BLA/MAA

- Adult Phase 1/2 full data
- ENERGY-3 full data
- ENERGY-1 available data
- ENERGY-2 available data
  - Natural history control group; patients matched on covariates associated with mortality

### Additional filings

- ENERGY-4 full data (Supplemental BLA/MAA)
- Japan, Brazil, Middle East

RGI-C: Radiographic Global Impression of Change, BMC/BMD: Bone mineral content/density, BLA: Biologics license application, MAA: Marketing authorisation application

*Supported by trends in appropriate secondary endpoints
**Plan to conduct this trial ex-U.S.. Discussions are ongoing with FDA regarding design of this trial in the U.S.
***Subject to regulatory discussions and appropriate financial resources

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# ABCC6 Deficiency

**Sienna**
Living with ABCC6 Deficiency

# ABCC6 Deficiency is a multisystem, rare genetic disease: High morbidity and a continuum of effects across age groups

## GACI-2
**0-1 Years**

***~10% mortality within 12 months of birth*** [1]

- Severe cardiovascular complications and pulmonary hypertension

## Pediatric
**1 to <18 years**

***Multisystem vasculopathy and strokes*** [2]

- Progressive cardiovascular calcification/stenosis of major arteries
- Cerebrovascular calcification - including stroke
- Initial retinal calcification

## PXE
**18+ Years**

***Blindness, cardiovascular disease and mobility impairment*** [3-7]

- Progressive arterial calcification
- Increased incidence of stroke and dementia
- Retinal calcification – Angioid streaks, atrophy
- Progressive calcification and fragmentation of elastic fibers

**Genetic Prevalence: 1:25,000 - 1:50,000** [8-9]

Sources: 1. Ferreira et al. JBMR 2021; 2. Grossi et al, Eur J Med Genet, 63 2020; 3. Shimada et al. Int.J.Mol.Sci. 2021; 4.Risseeuw et al. Retina, 2019; 5. Leftheriotis et al. J Vasc Surg, 2011; 6. Vanakker et al. Hum Mutat. 2008; 7. Van den Berg et al. Cerebrovasc Dis, 2000; 8. Internal, Unpublished Data; 9. Ferreira et al. Genet Med, 2021

# Completed Phase 1/2 trial of INZ-701 in adults with ABCC6 Deficiency successfully met all study objectives

## Safety

- INZ-701 demonstrated a **favorable safety profile**
- No serious or severe adverse events
- Low/moderate, sometimes transient, ADA titers

## PK/PD

- **Rapid** and **sustained increase in PPi** observed in highest dose cohort (1.8 mg/kg)

## Clinical

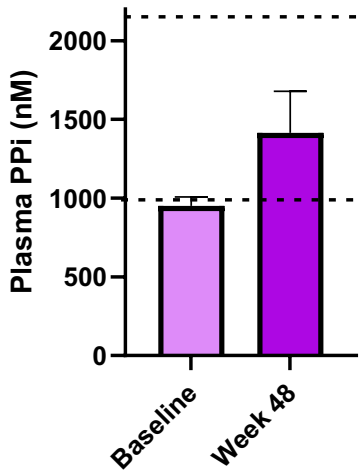
- **Positive changes** in multiple affected organ systems (cerebrovasculature and choroidal layer of eye) support **improvements in vascular health**
- Improvement in visual function (VFQ-25) and multiple PROs observed

VFQ-25: Visual Function Questionnaire; PRO: Patient Reported Outcomes; ADA: Anti-drug antibodies

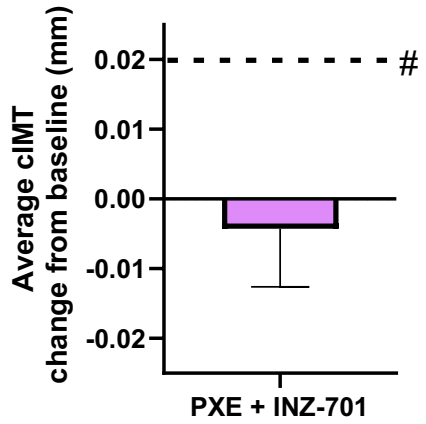
# INZ-701 showed benefit across multiple domains relevant for future pivotal trial

## Combined cohort 1-3 data comparing baseline to week 48

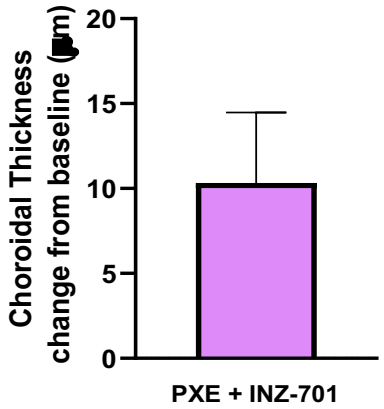
### PPi increased




### Carotid artery intima-media thickness decreased (cIMT)




### Choroidal thickness increased




– – – – – – Normal range

– – – – – – Mean annual cIMT change in TEMP study1

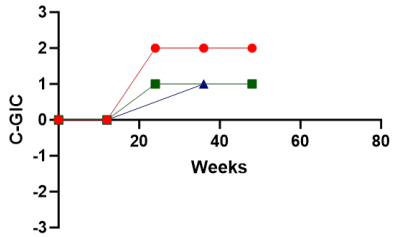
Data cut – 10 Jan 2024; Data presented as mean ± SEM Source: 1. Kranenburg et al, JACC, 2018

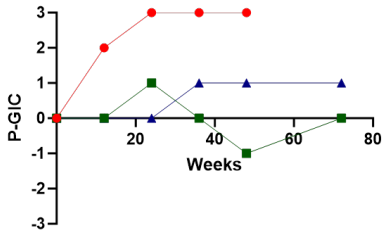
# Global Impression of Change Scale: Concordant improvement in C-GIC and P-GIC in all three dose cohorts

Clinician's Global Impression

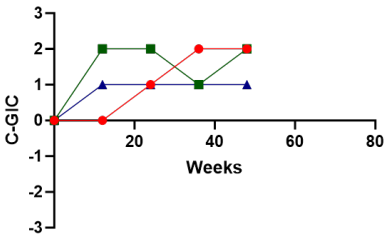
Patient's Global Impression

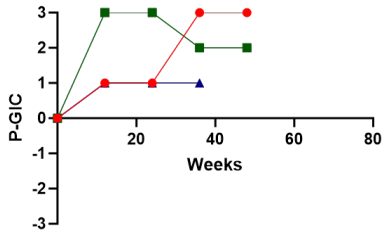
## Cohort 1 (0.2mg/kg) (n=3)



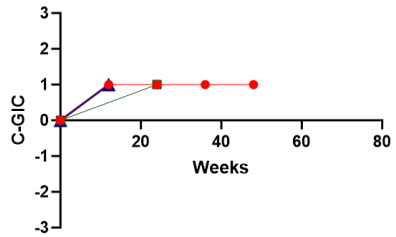



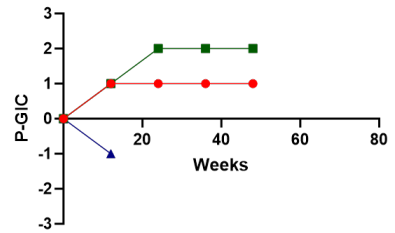

## Cohort 2 (0.6mg/kg) (n=3)




## Cohort 3 (1.8mg/kg) (n=3)




| -3 | -2 | -1 | 0 | 1 | 2 | 3 |
|---|---|---|---|---|---|---|
| Very much worse | Much worse | Minimally worse | No Change | Minimally improved | Much improved | Very Much improved |

Data cut – 10 Jan 2024

Colors represent individual patients in respective cohorts

# ABCC6 Deficiency: Planned Path to Global Approval

# Focused on pediatric population with ABCC6 Deficiency

## Unmet Need

- Retrospective natural history study (early-onset) and interventional study (adults) identified **risk of stroke** and **retinal disease** as consistent presentation in ABCC6 Deficiency

## Market

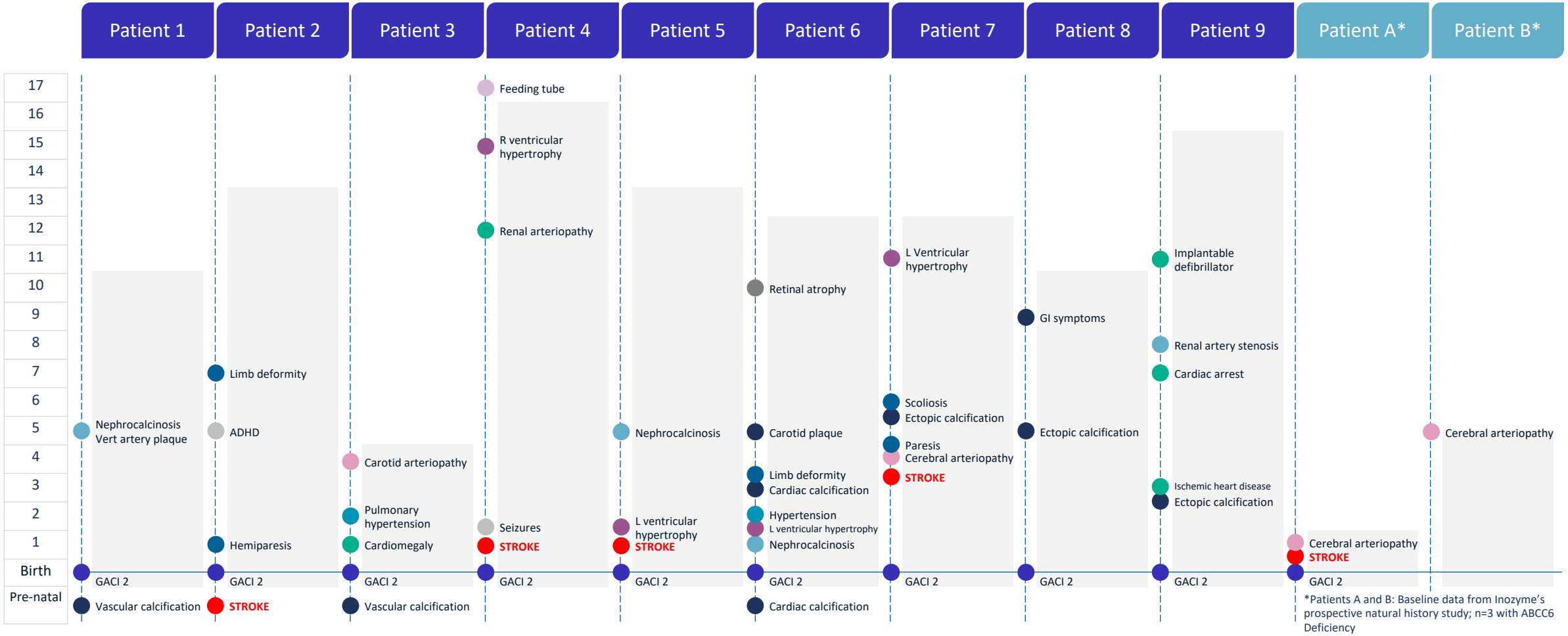
- Market research identified **substantial pediatric population** that represents the most important unmet need in ABCC6 Deficiency

## Regulatory

- Pivotal trial design planning in progress – potential for **approval** with primary and secondary endpoints selected based on serious clinical needs and disease pathology

VFQ-25: Visual Function Questionnaire; PRO: Patient Reported Outcomes; ADA: Anti-drug antibodies

# Retrospective Natural History Study: ABCC6 Deficiency patients had a heavy disease burden early in life




*Patients A and B: Baseline data from Inozyme's prospective natural history study; n=3 with ABCC6 Deficiency

Data cut – 10 Jan 2024

*Shaded area indicates age at time of informed consent*

# Planned roadmap for clinical development of INZ-701 in ABCC6 Deficiency

**ENERGY-1: Infant** (0-12 mos.)
Phase 1b
Single arm

**Pediatric (**≥1-<18 yrs.)*
Pivotal
Randomized, controlled

**Adult – PXE (18+)***
Pivotal
Randomized, controlled

**Adult – PXE (18+)**
Phase 1/2
Single arm – MAD

### *Ongoing Study*

- ***Safety*** *and* ***tolerability*** *as primary*
- ***PPi*** *and* ***survival*** *as secondary*

### *Future Studies*

- *Primary and secondary endpoints selected based on* ***serious clinical needs*** *and* ***disease pathology*** *(e.g., death, stroke, myocardial infarction, cardiac hospitalization, retinal disease progression, arterial calcification score, cIMT changes*
- *Composite endpoint comprised of* ***retinal measurements, peripheral arterial disease outcomes*** *and* ***PPi***

### *Completed Study*

- *Generally safe and well tolerated*
- *Consistently elevated PPi at highest dose*
- *Signals of clinical activity on vascular and ophthalmic for retinal endpoints*

## Basis for Planned Marketing Applications

**1st BLA/MAA**

- Adult Phase 1/2 full data
- ENERGY-1 available data
- Pediatric Pivotal trial data

**Additional filings**

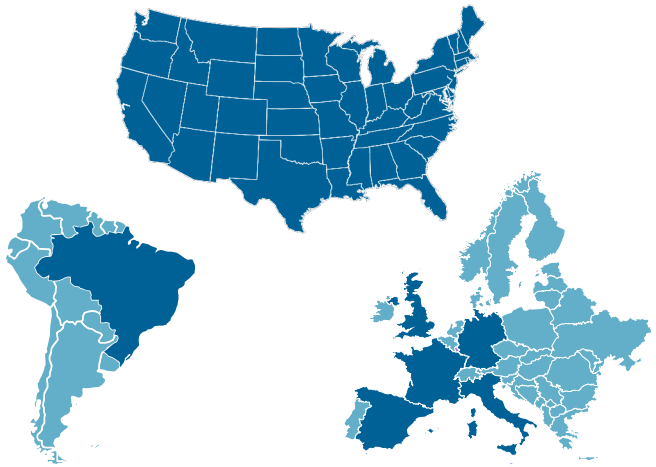
- Adult (18+) study (Supplemental BLA/MAA)
- Japan, Brazil, Middle East

*Subject to regulatory discussions and appropriate financial resources

# Building a Rare Disease Franchise

# Ongoing efforts to increase disease awareness, educate patient and medical communities, and improve access to genetic testing

## Growing Our Global Footprint



Currently evaluating Inozyme presence in Japan and Middle East

## Newborn Screening

**US – Rady Children's Hospital Network**
**UK – Genomics England**
Efforts ongoing to add to other panels across the globe

## Expanding HCP Audience

**Infant and Pediatric ENPP1/ABCC6**
- Fetal and pediatric cardiology
- Neonatology
- Pediatric endocrinology
- Maternal-fetal medicine
- Genetics

**Adult ENPP1/ABCC6**
- Endocrinology
- Nephrology
- Genetics
- Bone specialists

## Increasing Congress Attendance

NeoHeart – Cardiovascular Management of the Neonate
Society for Maternal-Fetal Medicine
PES Pediatric Endocrine Society
ASHG American Society of Human Genetics
ASBMR The American Society for Bone and Mineral Research
Human Growth Foundation
ECTS European Calcified Tissue Society
9th World Congress of Pediatric Cardiology and Cardiac Surgery

## Partnering to Remove Barriers to Diagnosis

GACI Global circulating hope
BeginNGS Newborn Genomic Sequencing to end the diagnostic odyssey
GENOMENON
ipm.ai Part of Real Chemistry
INVITAE
BlueDil int'l
Prevention Genetics
Genomics England
CENTOGENE
Rady Children's

# Identifying ENPP1 patients to support market potential – strong progress to date and expanding efforts into patients with monoallelic mutations

**Biallelic Genetic Prevalence[1]: 1:64,000**

**1000+** Global patients identified with **confirmed, known, or suspected ENPP1** Deficiency

| | | |
|---|---|---|
| **280+** | **Confirmed Patients** | Clinical and genetic diagnoses |
| **300+** | **Known Patients*** | Clinical diagnosis |
| **440+** | **Suspected Ped. Patients** | Identified using AI model of large US claims/EMR database |

*Internal data as of 1/4/24; number of confirmed patients expected to increase with patient/physician education, initiation of clinical trials, and progress towards potential regulatory approval*

* Phenotypic findings of disease only

### Identified ~82 confirmed symptomatic monoallelic ENPP1 patients

- Identified ~82 confirmed symptomatic monoallelic ENPP1 patients

### Majority of patients identified through Skeletal Disorders or Hypophosphatemia gene panels

- Suggests monoallelic patients can have clinical symptomatology similar to those with biallelic ENPP1 Deficiency

### Conducting observational study to characterize clinical features of adults with monoallelic ENPP1 mutations

Sources: 1. Ferreira et al. Orphanet Journal of Rare Diseases, 2022; 1:64,000 pregnancies

# ~1,300 likely U.S. pediatric ABCC6 Deficiency patients identified, representing ~70% of estimated genetic prevalence

## Pediatric ABCC6 Deficiency: U.S. Patient estimates

### Ischemic Stroke
**940 patients**

- *Ischemic stroke between ages 1-18*
- *Genetic panel ordered between ages 1 and <18 **OR** mild neurological symptoms occurred prior to stroke*
- *PXE or a phosphorous disorder diagnosis code in all history*
- *Exclusion of differential diagnoses*

### Angioid Streaks
**264 patients**

- *Angioid streaks between ages 1 and <18*
- *Exclusion of differential diagnoses and eye injuries*

### Retinal Imaging/OCT
**60 patients**

- *Optical coherence tomography (OCT) between ages 1 and <18*
- *Genetic panel ordered **AND** mild neurological symptoms occurred between ages 1 and <18*
- *PXE or a phosphorous disorder diagnosis code in all history*
- *Exclusion of differential diagnoses*

### Cardiovascular Anomaly
**24 patients**

- *Cardiovascular anomaly **AND** arterial calcification between ages 1-and <18*
- *PXE or a phosphorous disorder diagnosis code in all history*
- *Exclusion of differential diagnoses*

**Identified 1,288 likely U.S. pediatric patients with ABCC6 Deficiency**

Sources: IPM Longitudinal claims database An all-history (2009 – present) lookback period was utilized for clinical profiling Patients with 2+ claims for any one differential diagnosis (sickle cell disorders, Ehlers-Danlos syndrome, Paget's disease) were excluded. Based on 65% medical claims capture rate. No projection factor was used for claims coverage.

# Calciphylaxis

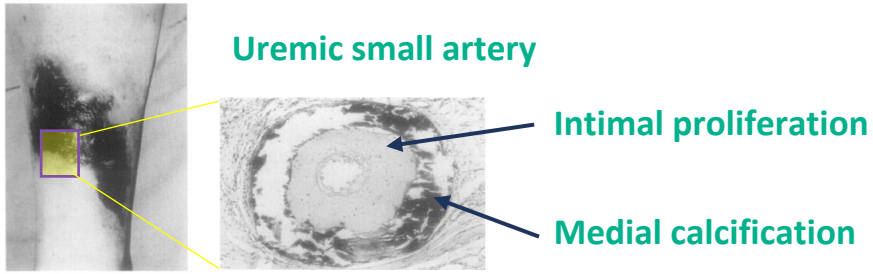
# Calciphylaxis: A severe complication of ESKD with high mortality and morbidity

## Calciphylaxis Incidence: 3.5 : 1,000 ESKD Patients[7]

**Major Markets Estimate: 5,000 patients/year**

Primarily affects end stage kidney disease (ESKD) patients[2]

### Vascular calcification-mediated disease

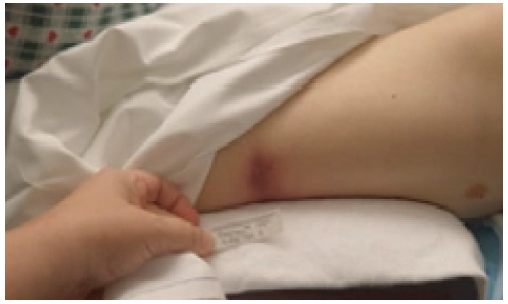



(Hafner et al, JAAD, 1995) (Hafner et al, JAAD, 1995)

Microvascular occlusion of skin arterioles caused by medial calcification, intimal proliferation, and thrombosis; Low PPi

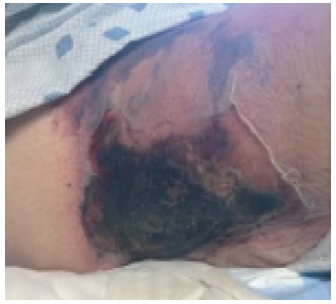
## Significant morbidity and mortality

Initial skin lesions typically present as extremely painful plaques and nodules, and progress to necrotic ulcers



Ghosh T, et al. Int J Dermatol. 2017

2 months



Ghosh T, et al. Int J Dermatol. 2017

- **>70%** require hospitalization for severe ulcerations[4]
- **~50%** of patients are bedridden or wheelchair-bound[4]

**~50% mortality**

**1 year after diagnosis[7]**

- Median survival time: 2.6 months[4]
- Sepsis most common cause of death[4-6]

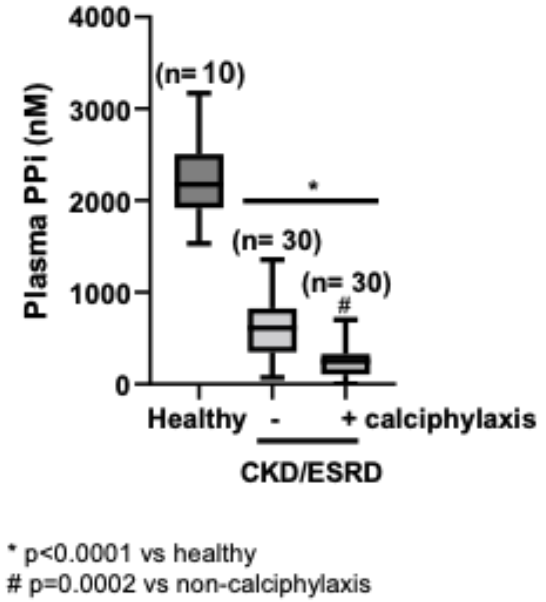
**No approved therapy**

Sources: 1. Nigwekar SU, et al. J Gen Intern Med. 2014; 2. Nigwekar SU, et al. J Am Soc Nephrol. 2016; 3. USRDS Annual Data Report 2021. https://adr.usrds.org/2021/end-stage-renal-disease/1-incidence-prevalence-patient-characteristics-and-treatment-modalities ;4. Weenig RH, et al. J Am Acad Dermatol. 2007; 5. Nigwekar SU, et al. Clin J Am Soc Nephrol. 2008; 6. Bazari H, et al. N Engl J Med. 2007; 7. Nigwekar et al. NEJM 2018.

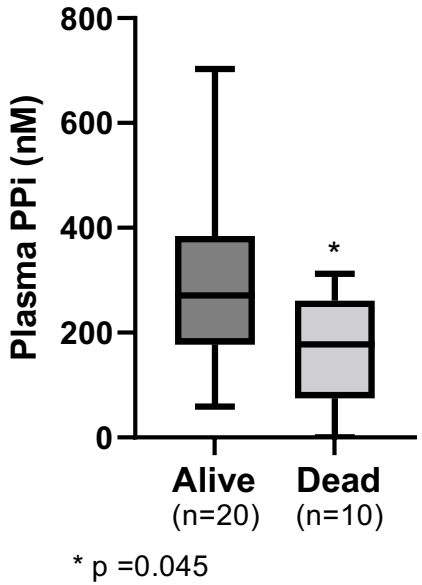
# Calciphylaxis is associated with PPi deficiency

Arteriolar calcification largely develops due to imbalance between calcification inhibitors and promoters[1-3]

**PPi levels lower in calciphylaxis**




**PPi levels predicted 6-month mortality among patients with calciphylaxis**




- ESKD patients had significantly lower PPi levels compared to healthy subjects[4]
- Calciphylaxis patients had significantly lower plasma PPi levels when compared with non-calciphylaxis ESKD patients[4]
- Published data showed correlation between PPi levels and severity of calcification

Data presented as median ± interquartile range

Sources: 1. Kramann R, et al. Nephrol Dial Transplant. 2013; 2. Moe SM, et al . Pediatr Nephrol. 2003; 3. Shroff R, et al. J Am Soc Nephrol. 2013; 4. Internal, Unpublished Data, ECTS 2022 Nigwekar et al; ESKD = End stage kidney disease, CKD = Chronic kidney disease

# SEAPORT-1: Phase 1 trial in patients with end-stage kidney disease (ESKD) receiving hemodialysis

Interim data expected in Q4 2024

**Study Population: Adults**

**Eligibility Criteria:**

- ≥18 to <70 years
- ESKD and receiving hemodialysis
- Undergoing 3 treatments of HD per week
- Low plasma PPi

**Design: Single arm, Open Label**

**n = up to 10**

**INZ-701 – 1.8 mg/kg Weekly**
coinciding with dialysis days

**30 days treatment, weekly dosing**

**Up to 3 US sites**

**Primary Goals**

- Change from baseline in **plasma PPi** concentration
- **Safety**

**Secondary Goals**

- **Pharmacokinetic (PK)** and **pharmacodynamic (PD)** parameters

RGI-C: Radiographic Global Impression of Change, RSS: Rickets Severity Score, PK: Pharmacokinetic, ARHR2: Autosomal Recessive Hypophosphatemic Rickets Type 2, ClinicalTrials.gov: NCT06046820

# Anticipated milestones provide robust news flow

| Milestone | 2024 | 2025 |
|---|---|---|
| **ENPP1 Deficiency** | | |
| • Topline Data – Adult Phase 1/2 Trial | ✓ | |
| • Initiate – ENERGY-2 Pivotal Trial in Infants – Ex. U.S. | 2H 24 | |
| • Interim Data – ENERGY-1 Phase 1 Infant Trial | 2H 24 | |
| • Topline Data – ENERGY-3 Pivotal Pediatric Trial | | Mid-Year |
| **ABCC6 Deficiency** | | |
| • Topline Data – Adult Phase 1/2 Trial | ✓ | |
| • Initiate Pivotal Trial* | | Q1 25 |
| **Calciphylaxis** | | |
| • Initiate SEAPORT-1 - Phase 1 Trial** | ✓ | |
| • Interim Data - SEAPORT-1 Phase 1 Trial | Q4 24 | |

*Pending regulatory discussions and appropriate financial resources, **Phase 1 trial in patients with end-stage kidney disease (ESKD) receiving hemodialysis

# Inozyme is at the forefront of developing novel therapies for rare diseases that affect bone health and blood vessel function

**ENPP1 Deficiency, ABCC6 Deficiency and calciphylaxis are serious diseases affecting bone health and blood vessel function linked to dysregulation of the PPi-Adenosine Pathway with no approved therapies**

**Lead product candidate INZ-701 demonstrated a rapid, significant, and sustained increase in PPi levels, preliminary evidence of efficacy, and a favorable safety profile across multiple clinical trials**

**Currently in pivotal trial for ENPP1 Deficiency; Completed Phase 2 trial for ABCC6 Deficiency and treatment period in Phase 1b trial in calciphylaxis program**

**Experienced team with a track record of success in rare disease**

**In a position of financial strength, with several expected upcoming milestones and a pipeline designed for long-term value creation**

- $166.2M expected to fund operations into Q4 2025 as of 3/31/24
- 61.85M common shares outstanding as of 5/2/24

# Thank you

**Ella**
Living with ENPP1 Deficiency