KOL & Patient Caregiver Webinar on Arginase 1 Deficiency

February 3, 2022

Nasdaq: AGLE

US-ARG-2200017

Forward Looking Statements & Disclosures

This presentation and the accompanying oral presentation contain "forward-looking" statements that are based on our management's beliefs and assumptions and on information currently available to management. Forward-looking statements include all statements other than statements of historical fact contained in this presentation, including information concerning our current and future financial performance, business plans and objectives, current and future clinical and preclinical development activities, timing and success of our ongoing and planned clinical trials and related data, the timing of announcements, updates, results of our clinical trials and related data and our planned BLA submission for our lead product candidate pegzilarginase, our ability to obtain and maintain regulatory approval, the potential therapeutic benefits, safety profile and economic value of pegzilarginase and our other product candidates, potential growth opportunities, financing plans, use and adequacy of financing plans, competitive position, industry environment and potential market opportunities.

Forward-looking statements are subject to known and unknown risks, uncertainties, assumptions and other factors including, but not limited to, those related to the success, cost and timing of our product candidate development activities and ongoing and planned clinical trials; our plans to develop and commercialize targeted therapeutics, including our lead product candidate pegzilarginase and our product candidates for the treatment of homocystinuria and cystinuria; the design, progress of patient enrollment and dosing in our clinical trials; the ability of our product candidates to achieve applicable endpoints in clinical trials; the safety profile of our product candidates in clinical trials; the potential for data from our current and future clinical trials to support a marketing application, as well as the timing of these events; the potential for preclinical studies to be predictive of current or future clinical trials; our ability to obtain funding for our operations, development and commercialization of our product candidates; the impact of the COVID-19 pandemic on our operations and clinical development activities, including on the timing of enrollment of our clinical trials; the timing of and our ability to obtain and maintain regulatory approvals; our ability to obtain regulatory approval for, and commercialize, pegzilarginase, and recognize milestone and royalty payments from our licensing and supply agreement with Immedica; the potential for expedited development and review of pegzilarginase as a result of its Breakthrough Therapy designation; the potential addressable markets of our product candidates; the rate and degree of market acceptance and clinical utility of our product candidates; the size and growth potential of the markets for our product candidates, and our ability to serve those markets; our commercialization, marketing and manufacturing capabilities and strategy; future agreements with third parties in connection with the potential commercialization of our product candidates; our expectations regarding our ability to obtain and maintain intellectual property protection; our dependence on third party manufacturers; our ability to develop our own commercial manufacturing facility; the success of competing therapies that are or may become available; our ability to attract and retain key scientific or management personnel; our ability to identify additional product candidates with significant commercial potential consistent with our commercial objectives; and our estimates regarding expenses, future revenue, capital requirements and needs for additional financing.

Moreover, we operate in a very competitive and rapidly changing environment, and new risks may emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties and assumptions, the forward-looking events and circumstances discussed herein may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements. Further information on these and other factors that could affect these forward-looking statements is contained in our most recent Quarterly Report on Form 10-Q filed with the U.S. Securities and Exchange Commission (SEC) and other reports filed by the SEC. You should not rely upon forward-looking statements as predictions of future events. Although our management believes that the expectations reflected in our forward-looking statements are reasonable, we cannot guarantee that the future results, levels of activity, performance or events and circumstances described in the forward-looking statements will be achieved or occur. We undertake no obligation to publicly update any forward-looking statements, whether written or oral, that may be made from time to time, whether as a result of new information, future developments or otherwise.

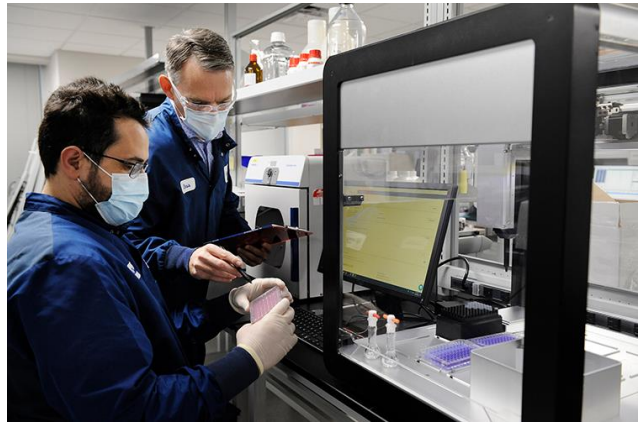
Dr. Barbara Burton and Dr. George Diaz have financial relationships with Aeglea BioTherapeutics, Inc. ("Aeglea"). Aeglea has also provided sponsorship funds to the Arginase 1 Deficiency Foundation, of which Tanja Brandt is an advisor and steering committee member. Ms. Brandt is also a member of the Aeglea Parent Caregiver Advisory Board and is compensated by Aeglea for her time participating in those activities.

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Addressing Metabolic Imbalances to Improve the Lives of Patients

Aeglea BioTherapeutics

At Aeglea, we believe that every patient deserves a chance at a better life. We are committed to helping people with rare and devastating metabolic diseases who have limited treatment options. Having a rare disease does not mean that you are in the fight alone.











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Agenda

- Welcome
- Overview of Arginase 1 Deficiency, Diagnosis and Role of Arginine
  - **Dr. Barbara Burton, M.D.,** Professor of Pediatrics, Northwestern University Feinberg School of Medicine, and Attending Physician in the Division of Genetics, Birth Defects and Metabolism at the Ann & Robert H. Lurie Children's Hospital of Chicago
- Enzyme Therapy Approach to Treating Arginase 1 Deficiency and Review of Clinical Data
  - **Dr. George Diaz, M.D., Ph.D.,** Professor of Genetics and Genomic Sciences, Icahn School of Medicine, and Director of the Program for Inherited Metabolic Diseases at Mount Sinai
- Living with Arginase 1 Deficiency, a caregiver perspective
  - **Tanja Brandt,** parent/caregiver of a 10-year-old child with ARG1-D, Advisor and Steering Committee Member of the Arginase 1 Deficiency Foundation (ARG1D.org)
- Q&A

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Overview of Arginase 1 Deficiency, Diagnosis and Role of Arginine

Barbara K. Burton, MD

Department of Pediatrics, Northwestern University Feinberg School of Medicine
Chicago, IL

Arginase 1 Deficiency (ARG1-D)

- Autosomal recessive disorder of arginine metabolism due to ARG1 enzyme deficiency
  - Recent genetic prevalence data suggests >2500 patients worldwide[1]
    - >700 patients in Europe
    - ≥250 patients in US
- Typically presents at ~2-4 years of age; can be later[2,3]
  - Developmental delay with increasing intellectual disability
  - Failure to thrive and short stature
- Prominent and progressive neurological manifestations[4]
  - Impaired mobility due to characteristic spastic diplegia
  - Seizures are a common feature
- May be initially misdiagnosed as cerebral palsy or hereditary spastic paraplegia[5]

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1. Aeglea Data on File. 2. Huemer M, et al. *J Inherit Metab Dis.* 2016;39:331–340. 3.Burrage LC, et al. *Hum Mol Genet*. 2015;24:6417–6427.
4. Carvalho DR, et al. *Pediatr Neurol.* 2012;46:369–374. 5.Carvalho DR et al. Gene 2012; 509(1): 124-130.

Arginase 1 Deficiency (ARG1-D)

- Additional abnormalities due to impairment of the urea cycle
  - Hyperammonemia but less prominent than other urea cycle disorders
  - Protein aversion, food refusal, and self-restriction of protein
- Low disease awareness leads to delays in diagnosis[1]
- Suspected cases can be diagnosed reliably
  - Raised Plasma arginine (>300 µM)[2]
  - Red blood cell arginase, genetic testing[3]
- Persistent high plasma arginine is accompanied by progressive and debilitating neurologic manifestations and functional impairment. Reducing plasma arginine improves manifestations even in patients with established disease[4]

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1. Huemer M, et al. J Inherit Metab Dis. 2016;39:331–340. 2. Haberle J et al J Inherit Metab Dis. 2019;42:1192-1230. 3. Diez-Fernandez C, et al. Hum Mutat. 2018;39:1029–1050. 4. Schlune A, et al Amino Acids. 2015;47:1751–1762.

ARG1-D: Diagnosis and Management

- Timely diagnosis and initiation of treatment to reduce arginine is essential to improving patient outcomes
  - Current standard of care for ARG1-D
    - dietary protein restriction
    - essential amino acid supplementation to minimize arginine intake while maintaining nutritional status
    - nitrogen scavengers to reduce risk of hospitalization owing to hyperammonemia[1]
  - Available management strategies for ARG1-D rarely achieve recommended plasma arginine levels[2]
  - Patients continue to experience significant morbidity but reduction of arginine and cumulative arginine toxicity is important for delaying or diminishing progression of manifestations and may promote clinical stabilization

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1. Haberle J et al J Inherit Metab Dis. 2019;42:1192-1230. 5. Burrage LC, et al. Hum Mol Genet. 2015;24(22): 6417-6427.

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Case Study

Presented at the 14th International Congress of Inborn Errors of Metabolism

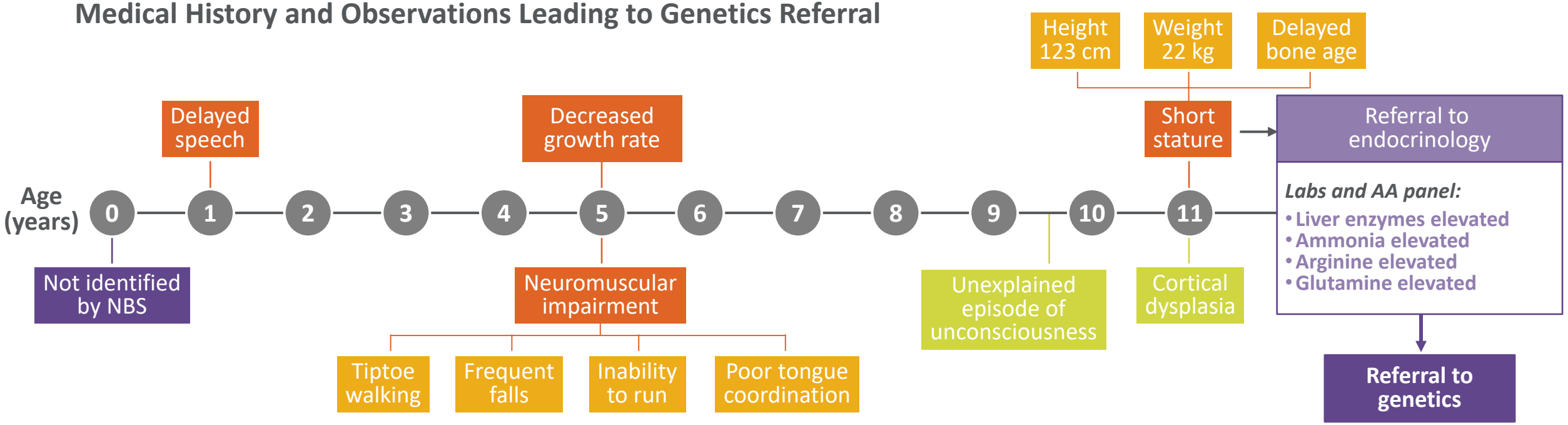
November 21–23, 2021 (Sydney, Australia)

Patient Medical History (Birth to Genetics Referral)

- This case describes a Hispanic female who was diagnosed with ARG1-D at 11 years of age after referral to genetics and who has since been followed for nearly 15 years
- The patient's medical history and clinical profile upon presentation led to initial suspicion of cerebral palsy. Observation of small stature led to referral to endocrinology, and after comprehensive biochemical testing the patient was referred to genetics (Figure, next slide)
  - The patient was born in Ecuador and not identified by newborn screening, likely because of limited access or program implementation
  - Delayed speech was observed at 12–13 months of age. By age 5 years, decreased growth and multiple indicators of neuromuscular impairment were evident
  - Suspicion of a metabolic disorder and subsequent genetics referral occurred after basic labs and amino acid testing revealed abnormalities consistent with a urea cycle disorder

Patient Medical History (Birth to Genetics Referral)

Medical History and Observations Leading to Genetics Referral



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AA, amino acid; NBS, newborn screening.

ARG1-D Diagnosis

- Presence of lower-limb hyperreflexia and tight heel cords, along with other neuromuscular manifestations, intellectual disability, and short stature were felt to be consistent with either cerebral palsy or a metabolic disorder. Biochemical and genetic testing was pursued to reach a definitive diagnosis (Figure, next slide)
  - At the time of presentation to genetics, the patient's plasma arginine was nearly 6-fold the upper limit of normal, indicating a likely diagnosis of ARG1-D. Additional biochemical observations consistent with ARG1-D included slightly elevated glutamine and mild hyperammonemia (Table, next slide)
  - Enzyme assay revealed red blood cell arginase activity of 0.1 µmol/h/mg hemoglobin (normal range, 2–7 µmol/h/mg), further supporting a likely diagnosis of ARG1-D
  - Genetic sequencing revealed a homozygous novel *ARG1* variant (c.371-A>G; active site D124G, predicted deleterious), confirming a definitive diagnosis of ARG1-D

Diagnostic Methods and Biochemistry Labs

Diagnostic Testing

High pArg:
621.2 µmol/L

Low arginase activity:
0.1 µmol/h/mg Hgb

***ARG1* novel variant:**
c.371-a>g
(predicted deleterious)

Relevant Biochemistry Before Treatment vs After 14 years on Treatment

Analyte (normal range)	Pre-treatment (Jul – Aug 2007)	Last on-treatment follow-up (Aug 2021)
Liver enzymes		
ALT (2–30 µmol/L)	130	Normal
AST (11–35 µmol/L)	67	Normal
ALP (60–320 IU/L)	402	Normal
Key amino acids*		
Arginine (40-115 µmol/L)	527 656 681	420
Glutamine (254–823 µmol/L)	884 999 875	648
Ammonia (11-35 µmol/L)	48	67

*Amino acid panels were run 3 times from July to August 2007.

- Upon diagnosis, standard of care consisting of **dietary protein restriction with essential amino acid supplementation** and nitrogen scavenger therapy was initiated

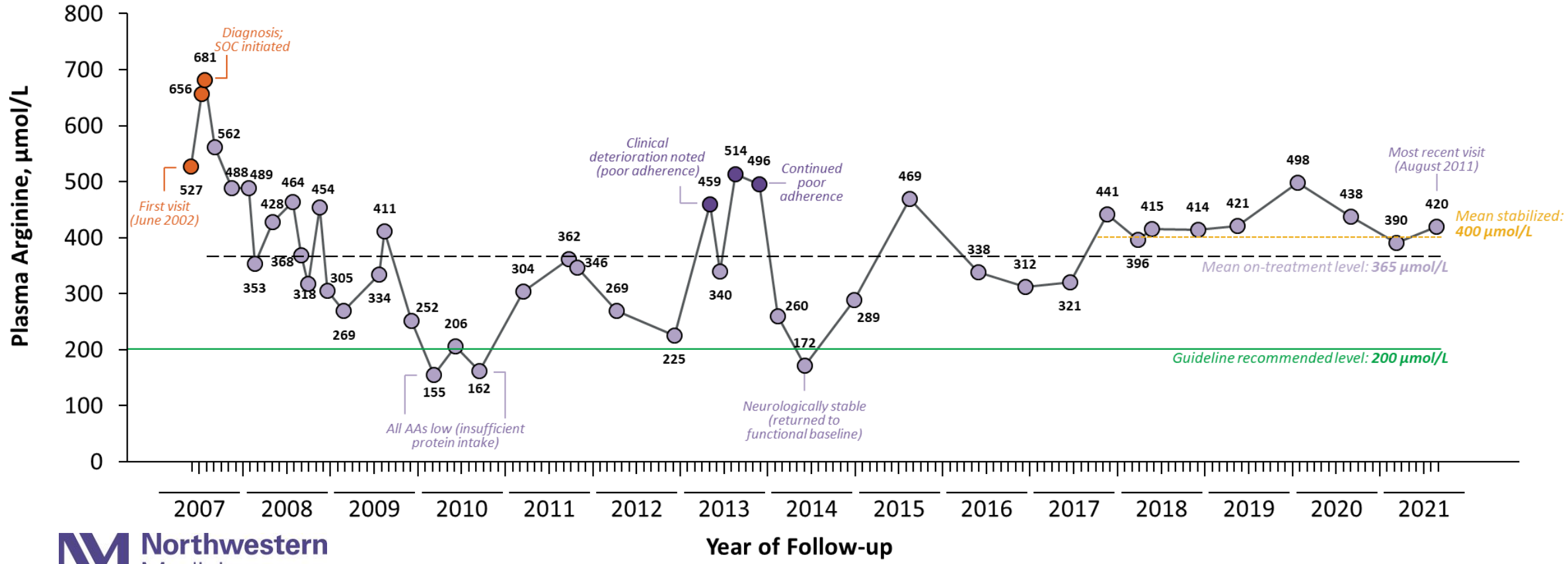
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ALT, alanine transaminase; AST, aspartate transaminase; ALP, alkaline phosphatase.

Long-Term Outcomes

- Plasma arginine has been lowered from pretreatment levels but remains high and was more than 2× the recommended level of ≤200 µmol/L[1] at the most recent visit (420 µmol/L; Figure)

Long-Term Plasma Arginine Time-Course



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1. Haberle J et al J Inherit Metab Dis. 2019;42:1192-1230.

Long-Term Outcomes *(continued)*

- At >14 years of follow-up, the patient, now 25 years of age, is generally clinically stable and remains both verbal and ambulatory (Table); however:
  - pArg remains persistently high
  - Cognitive impairment has persisted and progressed; the patient cannot live independently
  - Neuromuscular involvement in the form of toe walking and spasticity has persisted and varies in severity despite introduction of baclofen and botulinum toxin

Profile	Long-term Treatment Outcomes
Overall	• Generally stable
Biochemical	• Liver enzymes normalized • **Arginine remains markedly elevated (~400 µmol/L)** • Ammonia is variable
Cognitive	• Remains verbal • **Persistent, evolving impairment**
Neuromuscular/mobility	• Remains ambulatory • **Persistent gait abnormalities** • **Persistent spasticity**

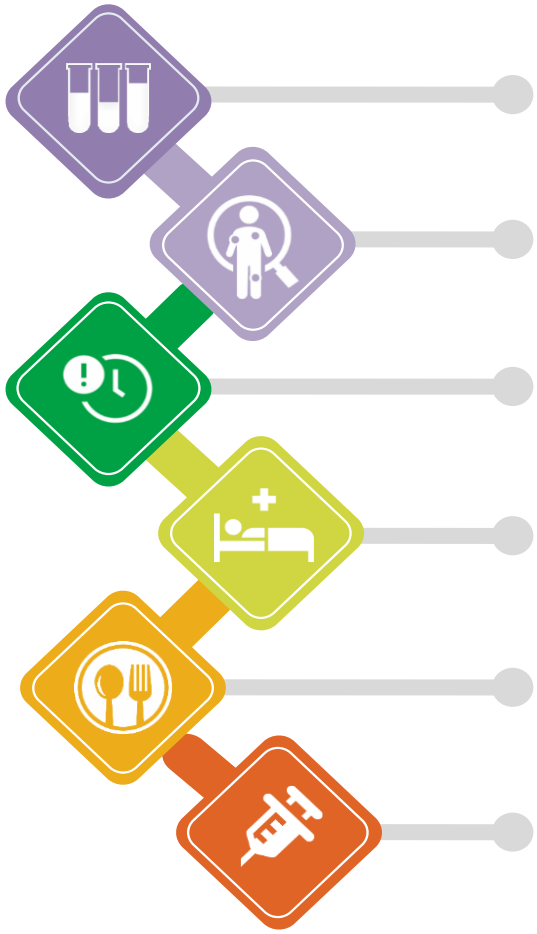
Summary and Conclusions

- The patient presented with manifestations and medical history consistent with ARG1-D but also common in cerebral palsy, which was initially suspected. Short stature and a complex clinical profile prompted further evaluation, and the patient was quickly and definitively diagnosed with ARG1-D through biochemical and genetic testing
- Chronic high arginine is known to lead to progression of cognitive and neuromuscular manifestations in ARG1-D. Occurrence of acute clinical deterioration associated with transient increases in plasma arginine further demonstrates the role of arginine as the driver of these manifestations
- Early diagnosis and initiation of treatment to lower plasma arginine is critical for decreasing or delaying progressive worsening of manifestations
- **The current standard of care does not adequately lower plasma arginine, as illustrated by persistent high levels and continued progression for this patient, and there is an urgent need for more effective interventions**

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1. Huemer M, et al. J Inherit Metab Dis. 2016;39:331–340. 2. Haberle J et al J Inherit Metab Dis. 2019;42:1192-1230.
3. Diez-Fernandez C, et al. Hum Mutat. 2018;39:1029–1050,

ARG1-D is a Debilitating Metabolic Disease that Severely Impairs Patients' Quality of Life



ARG1-D is **a progressive, debilitating, metabolic disease** characterized by high levels arginine[1,2]

In its classic presentation, three major manifestations begin in early childhood: **spasticity, seizures, and intellectual disability**[2–4]

Delays in diagnosis are common, leading to disease progression and worse clinical outcomes[5]

Burden on patients and caregivers is high, and most patients lose the ability to walk independently[2]

Only treatment is severe dietary protein restriction, which commonly does not reduce arginine levels to current goal of <200 µmol/L[2,3,6]

New and effective therapies are needed

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1. Carvalho DR, et al. *Gene.* 2012;509:124–130. 2. Carvalho DR, et al. *Pediatr Neurol.* 2012;46:369–374. 3. Amayreh W, et al. *Dev Med Child Neurol*. 2014;56:1021–1024. 4. Bakhiet M, et al. *Medicine (Baltimore).* 2018;97:e10780. 5. Huemer M, et al. *J Inherit Metab Dis.* 2016;39:331–340. 6. Haberle J, et al. *Orphanet J Rare Dis.* 2012;7:32.

Thank You

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Enzyme Therapy Approach to Treating Arginase 1 Deficiency and Review of Clinical Data

George A. Diaz, MD, PhD

Division of Medical Genetics and Genomics
Department of Genetics & Genomic Sciences
Icahn School of Medicine at Mount Sinai



Arginase 1 Deficiency (ARG1-D)

ARG1-D is a serious, progressive disease with early mortality and high unmet medical need. It is caused by a mutation in the arginase 1 enzyme, resulting in persistently high levels of arginine.

Clinical Presentation

- Diagnosed age 1 after presentation with hyperammonemia
- Developed severe lower limb spasticity, speech delay, intellectual disability
- Treated with severe protein restriction, essential amino acids, and ammonia scavengers
- Progressive worsening of lower extremity spastic diplegia, ambulates with arm crutches
- C.371A>G (single point active site mutation D124G)
- Arginase activity 0%



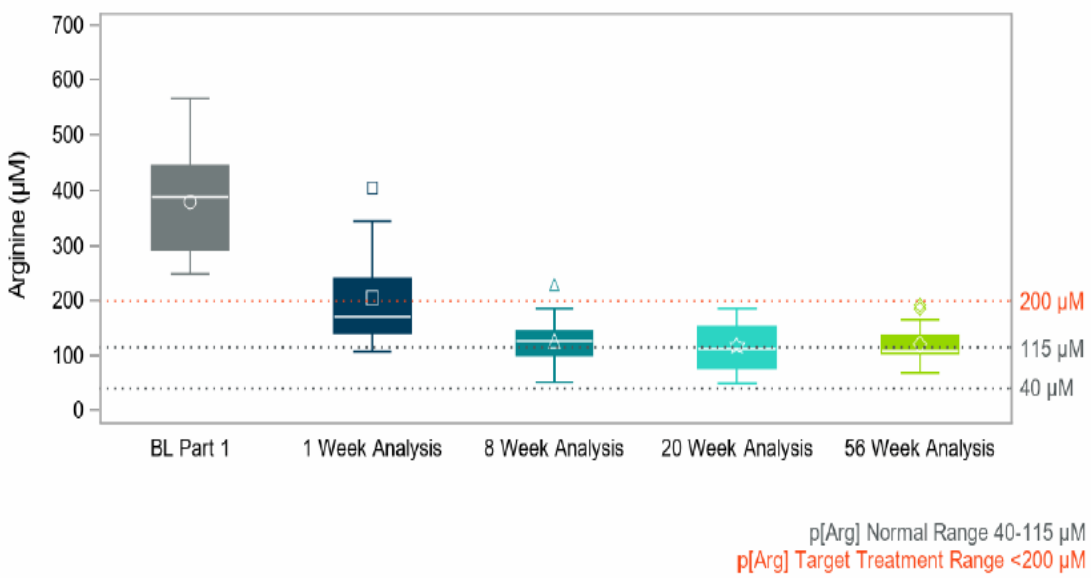
Assessment	Normal Population	Patient Baseline
Plasma arginine	40 – 115 µM	**389 µM**
6MWT	Reference Interval: 310 – 664 meters	**174 meters**
GMFM-E	Max = 72	**27**

[1] Diez-Fernandez C, et al. Hum Mutat. 2018;39:1029-1050. [2] Carvalho DR, et al. Pediatr Neurol. 2012;46:369-374. [3] Crombez EA, Cederbaum SD. Mol Genet Metab. 2005;84:243-251. [4] De Deyn PP, et al. Hyperargininemia: a treatable inborn error of metabolism. In: Guanidino Compounds in Biology and Medicine. London, UK: John Libbey Company Ltd.; 1997:53-69. [5] Prasad A, et al. J Child Neurol. 1997;12:301-309. [6] Amayreh W, et al. Dev Med Child Neurol. 2014;56:1021-1024. [7] Scaglia F, Lee B. Am J Med Genet C Semin Med Genet. 2006;142C:113-120. [8] Sin YY, et al. J Mol Med (Berl). 2015;93:1287-1296. [9] Cai X, et al. Medicine (Baltimore). 2018;97:e9880. [10] Schlune A, et al. J. Amino Acids. 2015;47:1751-1762. [11] Sun A, et al. Arginase deficiency. In: Adam MP, et al, eds. GeneReviews®. Seattle, WA: University of Washington, Seattle; 2020. [12] Diaz GA, et al. Poster presented at: 13th European Paediatric Neurology Society (EPNS) Congress; September 17-21, 2019; Athens, Greece. Poster P06-34.

Pegzilarginase Program Overview

Pegzilarginase is a novel recombinant human enzyme engineered to lower arginine levels

Plasma Arginine in Response to Pegzilarginase in Phase 1/2 & OLE Studies



First Clinical Program Ever Conducted in ARG1-D

Phase 1/2 and Open-Label Extension Trials

- 13 patients remain on therapy from 2-4 years
- Arginine lowering was rapid and durable
- Improvements in functional measures sustained over time

PEACE Phase 3 Clinical Trial

- First placebo-controlled trial ever conducted in ARG1-D
- Topline announced December 2021
  - Pegzilarginase reduced arginine levels by 80%, normalized arginine levels in 90.5% of patients
  - Positive trend in mobility measure
  - Well-tolerated
- 31 patients enrolled in long-term extension study

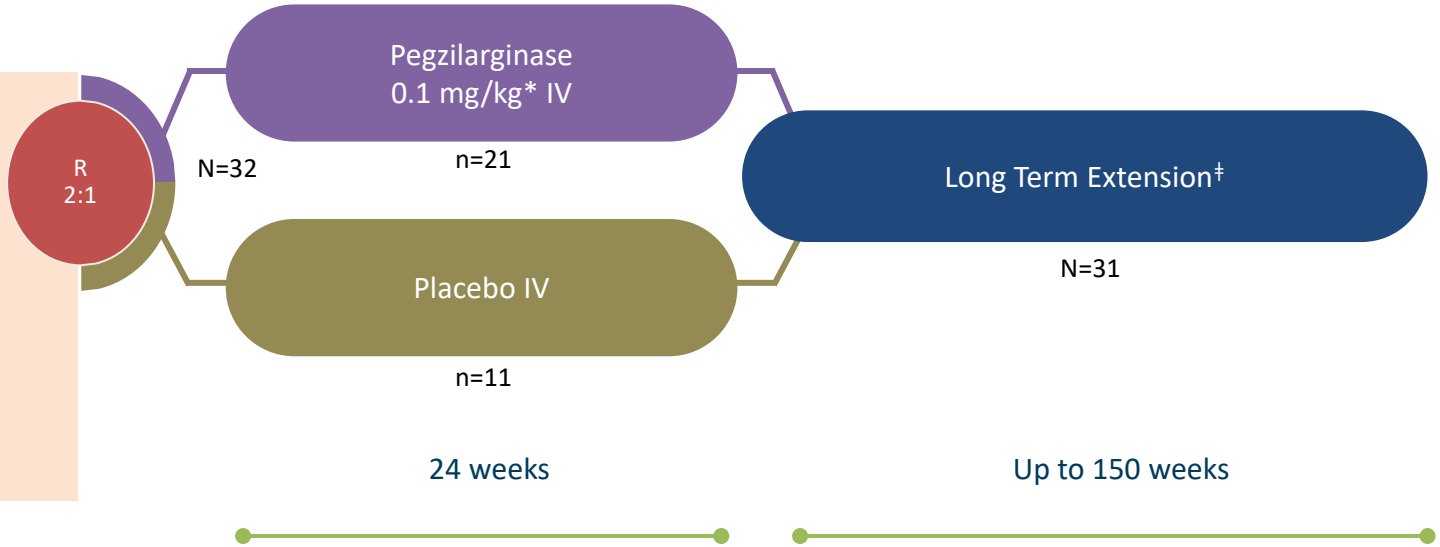
Pegzilarginase is an investigational drug and has not been approved by the U.S. Food and Drug Administration

PEACE Phase 3 Study Design

Double-blind portion of trial complete – topline data announced December 2021

Patients with ARG1-D

- ≥2 years old
- Plasma arginine >250 µM (mean)
- Baseline deficit in clinical response assessments



Key Endpoints	Analysis
Primary: • **Plasma arginine reduction**	• Reduction of plasma arginine compared to placebo
Secondary: • **GMFM-E (walking, running & jumping)** • **2-Minute Walk Test (distance walked in 2 min)**	• Continuous analysis • Improvement compared to placebo

*Dosing is weekly and, if needed, dose is modified based on plasma arginine levels with maintenance of blinding.
‡The first 8 weeks of the open-label extension will be blinded. All study participants remain on current disease management for the duration of the trial. Dose adjustments in the double-blind treatment period can be made to optimize plasma arginine control for levels outside the range of 50-150µM. If needed, weekly doses can be increased to 0.15 and 0.2 mg/kg or reduced to 0.05mg/kg
ARG1-D = Arginase 1 Deficiency; IV = intravenous; R = randomized.

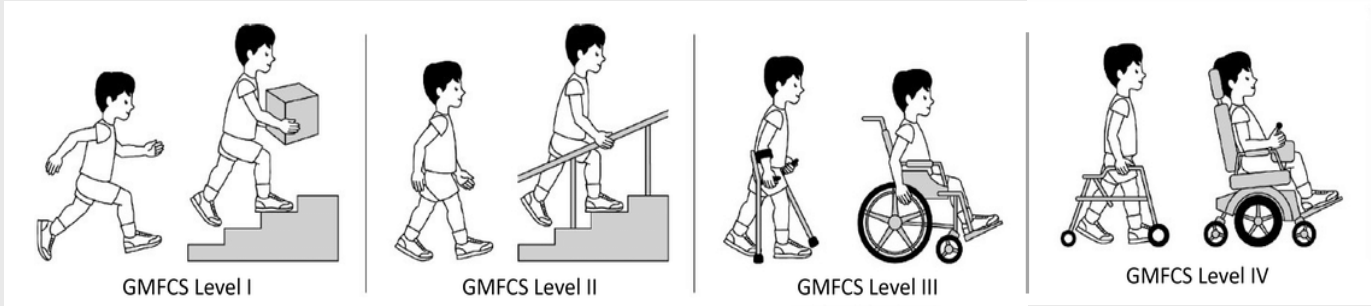
Measuring the Functional Impact of Spasticity in ARG1-D

Classification of Mobility:

Gross Motor Function Classification System (GMFCS)

Overall description of current motor function based on:

- Movements such as sitting, walking
- Use of mobility devices



Level V not pictured: adapted seating and assistance with transfers, and utilize wheeled power mobility independently or manual mobility with assistance in most settings

Measures of Change in Mobility:

Gross Motor Function Measure (GMFM) Part E

Assesses **unaided mobility** without bracing or assistive devices

- 24 tasks involving walking forward/backward, running, jumping and ascending/descending stairs with a score range of 0-72





Timed Walk Tests

Evaluates **aided mobility** with any needed bracing or assistive devices over a defined period of time

- 2 mins (2MWT)
- 6 mins (6MWT)



GMFCS graphic adapted with permission from The Royal Children's Hospital Melbourne, Australia

Pegzilarginase Safety Profile – Results from PEACE

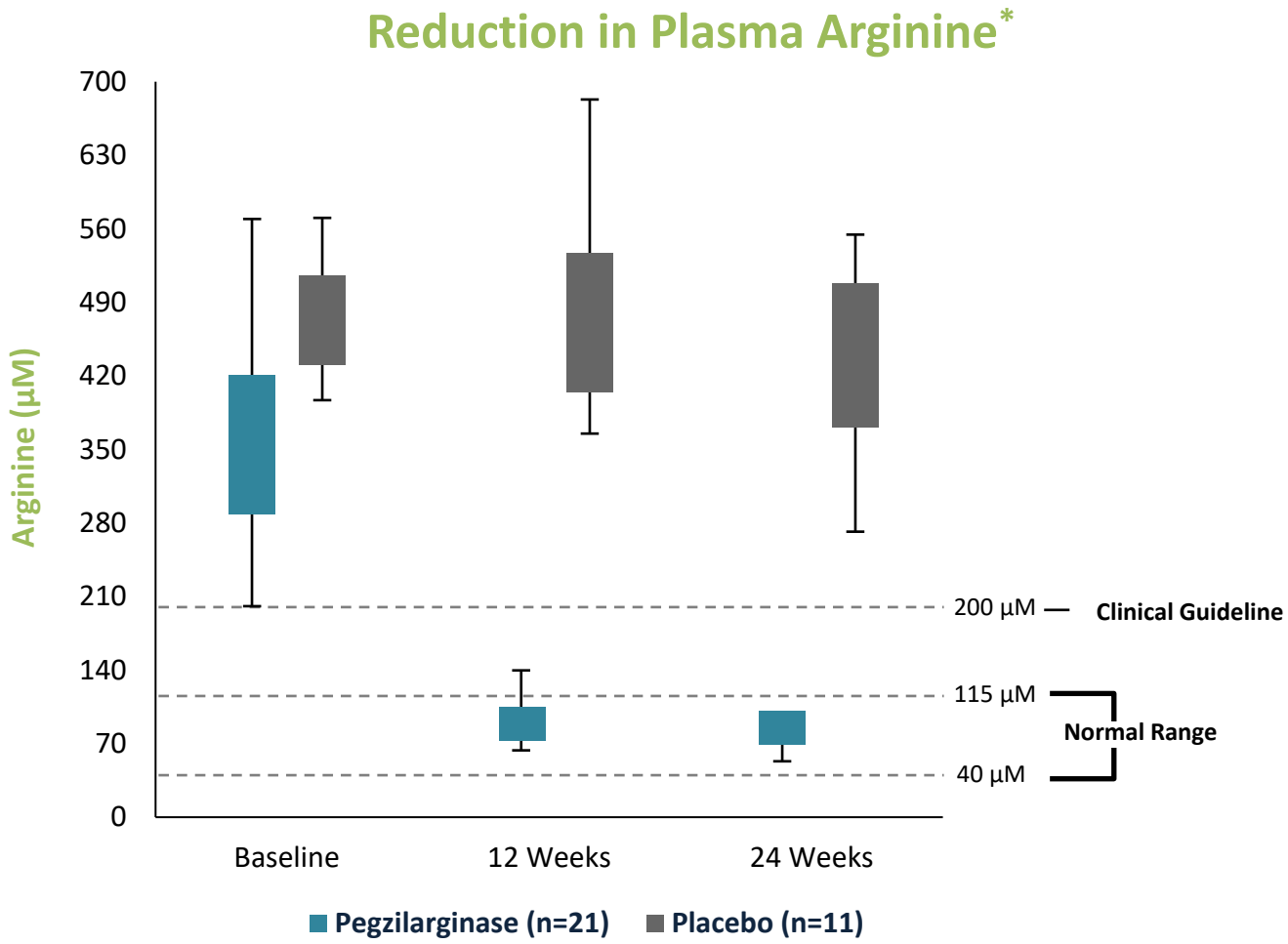
Patients With:	Pegzilarginase n (%)	Placebo n (%)
Any TEAE *	18 (85.7)	11 (100.0)
TEAE Leading to Discontinuation	0	0
SAE ‡	4 (19.0)	4 (36.4)
Hypersensitivity Reaction ◊	2 (9.5)	0
Hyperammonemia ◊	3 (14.3)	4 (36.4)

- No discontinuations due to TEAEs
- Most TEAEs were mild or moderate in severity
- Common TEAEs occurring in >15% of patients in treatment and placebo arms included: **vomiting, cough, hyperammonemia, nausea, abdominal pain, fever, decreased appetite**
  - With exception of vomiting, cough, and fever, each event occurred at lower rate compared to placebo
- Hypersensitivity reactions were infrequent (<10% of patients) and managed with routine medical care
- SAEs included hyperammonemia and vomiting (1 patient)

Pegzilarginase was Well-tolerated with No Discontinuations in Pivotal Study Due to Adverse Events

* TEAE: Treatment-Emergent Adverse Event; ‡ SAE: Serious Adverse Event; ◊ Adverse Event of Interest

Primary Endpoint Met – Marked and Sustained Reduction in Plasma Arginine



- Highly elevated plasma arginine despite standard of care
- Mean plasma arginine*
  - Pegzilarginase
    - Baseline: 365.4 µM
    - 24-week: 105.5 µM
  - Placebo
    - Baseline: 471.7 µM
    - 24-week: 448.8 µM

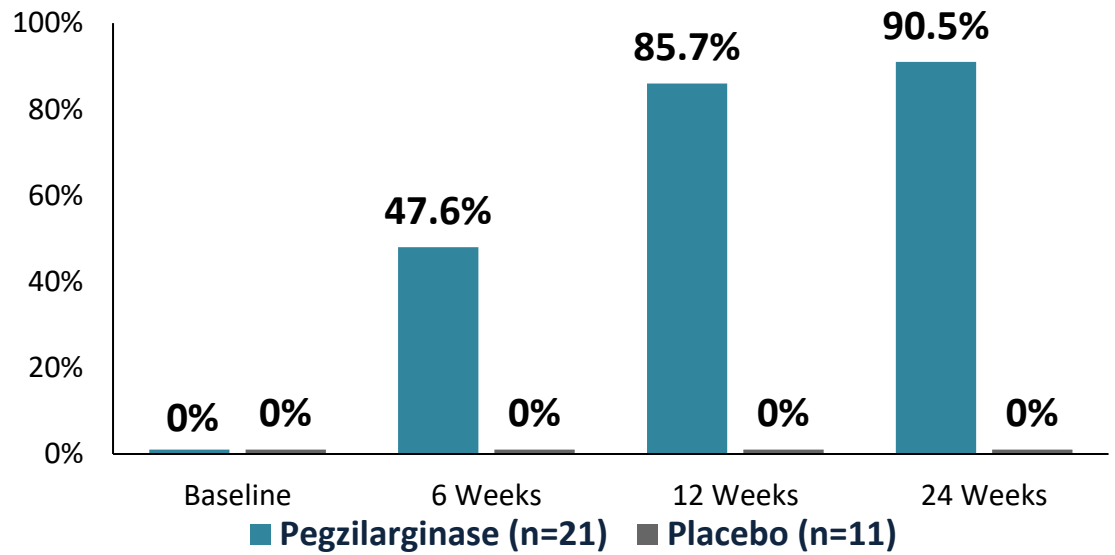
Primary Endpoint – 24-Week Analysis

- **80% reduction in mean plasma arginine with pegzilarginase ($p<0.0001$)‡**

*Based on arithmetic mean of values from visit and preceding 3 visits, boxes represent middle 50% with error bars depicting 95% confidence interval; ‡ Statistical analysis based on geometric mean`

Normalization of Plasma Arginine Levels in ARG1-D Patients

Proportion of Patients Reaching Normal Arginine Levels (40-115 µM)



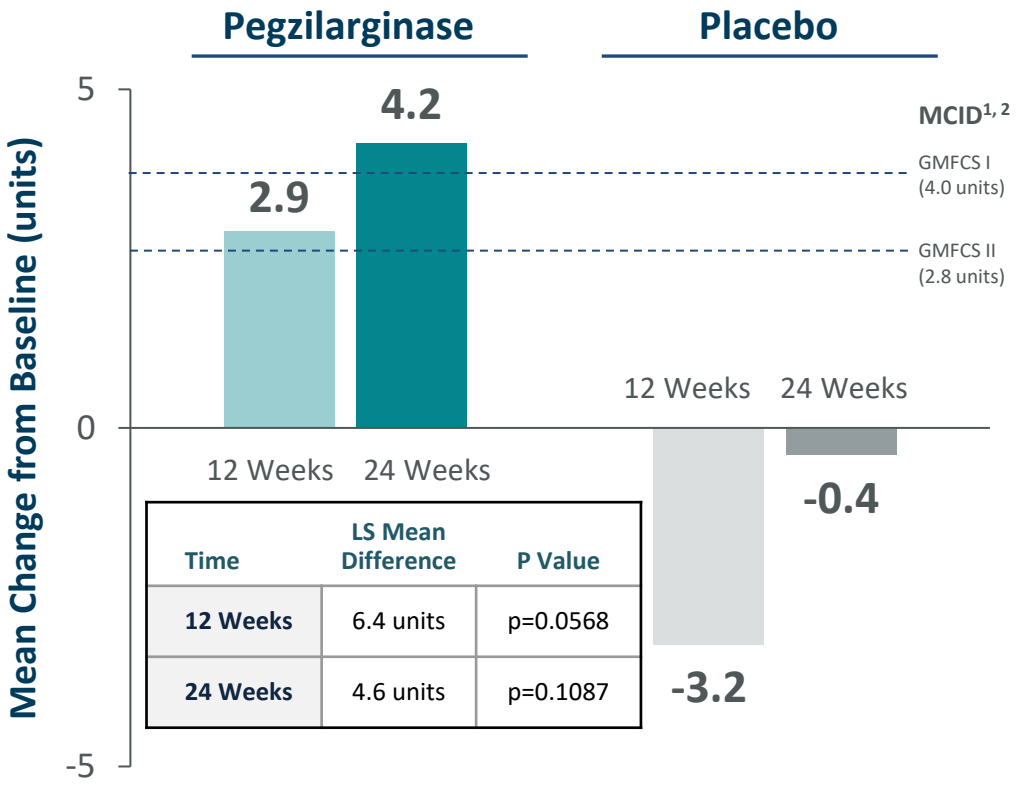
- 90.5% of pegzilarginase treated patients achieved normal plasma arginine levels ($p<0.0001$)*
- No meaningful change in plasma arginine levels in placebo arm
- Treatment with pegzilarginase surpassed current clinical guideline goals of reducing arginine levels below 200 µM

Potential Transformative Therapy for a Patient Population with Previously Unachievable Control of Arginine to Normal Levels

* Nominal p value, based on arithmetic mean of values from visit and preceding 3 visits

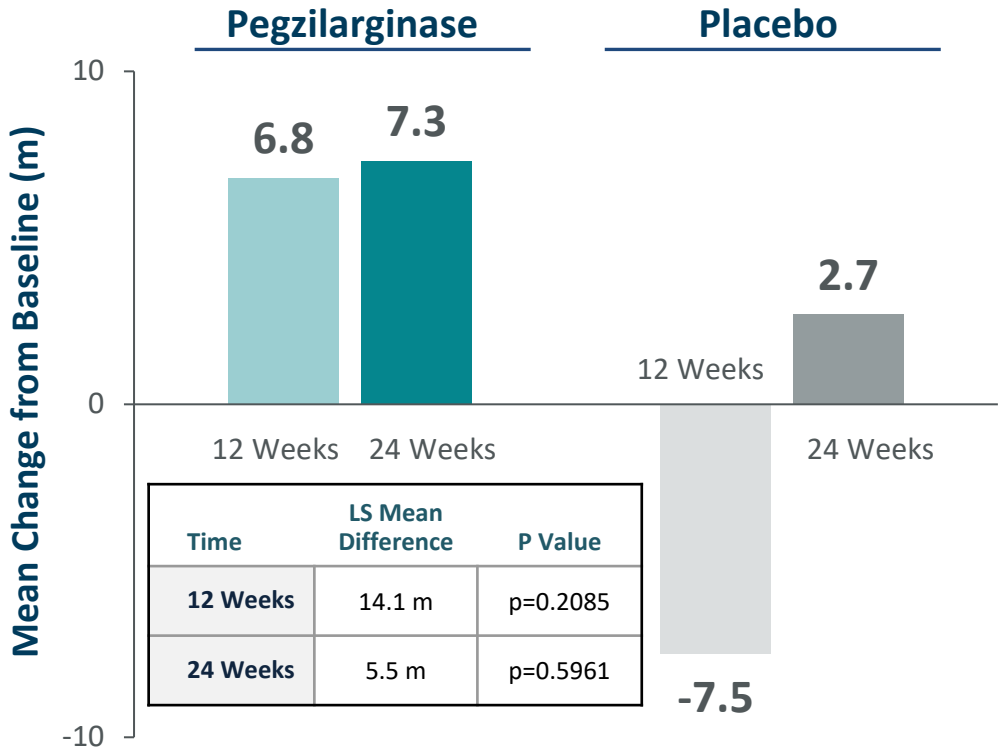
PEACE Mobility Assessed by GMFM-E and 2MWT

Mean Change from Baseline in GMFM-E



Time	LS Mean Difference	P Value
12 Weeks	6.4 units	p=0.0568
24 Weeks	4.6 units	p=0.1087

Mean Change from Baseline in 2MWT



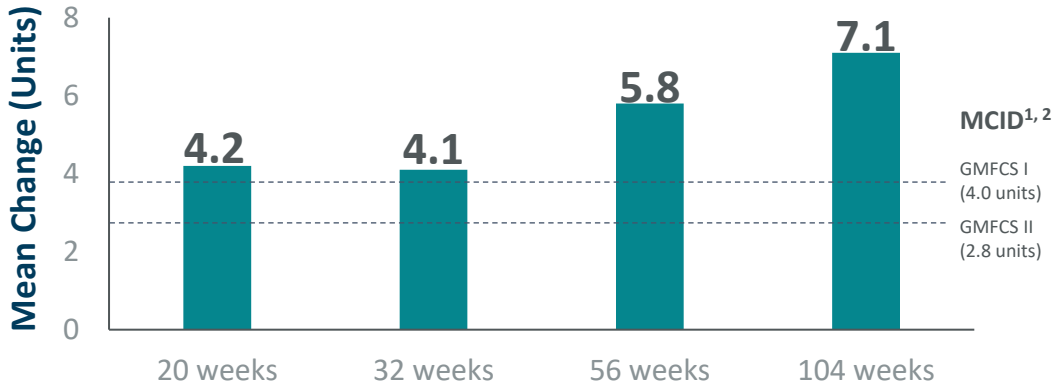
Time	LS Mean Difference	P Value
12 Weeks	14.1 m	p=0.2085
24 Weeks	5.5 m	p=0.5961

Positive Trend in GMFM-E in Placebo-Controlled Study Suggests Potential Improvements in Mobility

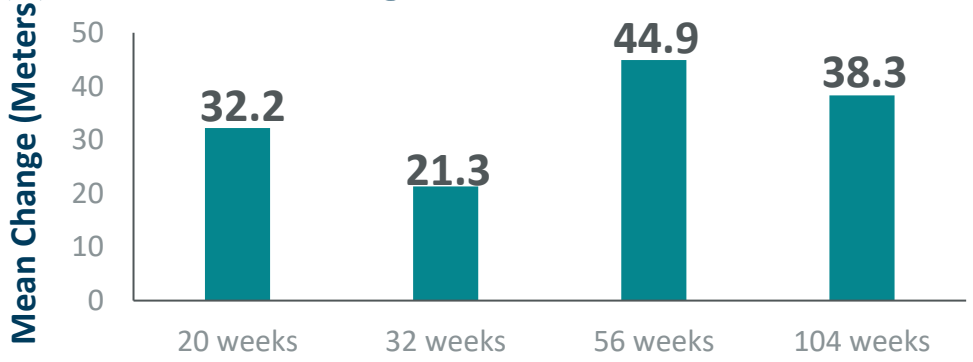
[1] MCID is minimal clinically important difference defined in Cerebral Palsy population. [2]Oeffinger D et al 2008; GMFM-E = Gross Motor Function Measure Part E; 2MWT = 2-minute walk test; GMFCS = Gross Motor Function Classification System

Improvements in Mobility Measures Sustained Over Time in Phase 1/2 and OLE

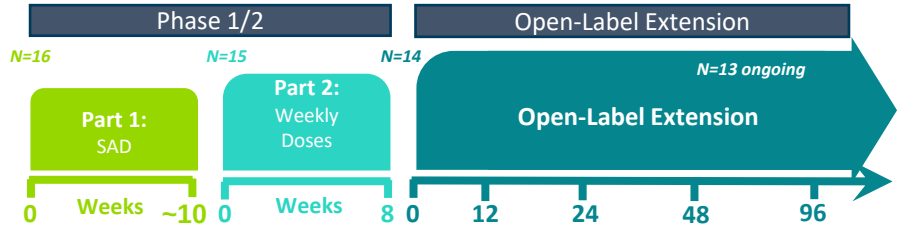
Mean Change from Baseline in GMFM-E



Mean Change from Baseline in 6MWT



Endpoint	Change from Baseline for Phase 1/2 & OLE Studies[3]			
	20-week analysis (N=13)	32-week analysis (N=13)	56-week analysis (N=13)	104-week analysis (N=9)[4]
GMFM-E	4.2 units	4.1 units	5.8 units	7.1 units
6MWT	32.2 m	21.3 m	44.9 m	38.3 m



[1] MCID is minimal clinically important difference defined in Cerebral Palsy population. [2]Oeffinger D et al 2008; [3] Analysis weeks are calculated by adding the 8 weeks from Part 2 of the Phase 1/2 study to the weeks of treatment in the open-label extension (OLE) study (e.g., 8 weeks of Part 2 + 12 weeks of OLE is the 20-week analysis), baseline values were taken at Part 1 of the study; [4] 13 patients remain in the OLE study, 9 of 13 patients had 96-week mobility assessments conducted; GMFM-E = Gross Motor Function Measure Part E; 6MWT = 6-minute walk test; GMFCS = Gross Motor Function Classification System; SAD = single ascending dose

Summary of Findings and Future Impact

PEACE Study

- The first ever placebo-controlled trial in Arginase 1 Deficiency
- Confirmed that arginine levels were persistently elevated in spite of being on standard of care

Clinically Meaningful Results

- Normalized arginine levels which was previously unachievable in this patient population
- GMFM-E trend highly suggestive of mobility benefit with improvement over time
  - Mean changes in both trials greater than MCID of 4 units and 2.8 units for GMFCS Levels I and II respectively[1]
- Strong, consistent data





Pegzilarginase has the Potential to be a Life-Changing Therapy for ARG1-D Patients and Their Families

[1] Oeffinger D et al 2008; MCID = minimal clinically important difference; GMFCS = Gross Motor Function Classification System

Thank You



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Q&A



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US-ARG-2200017