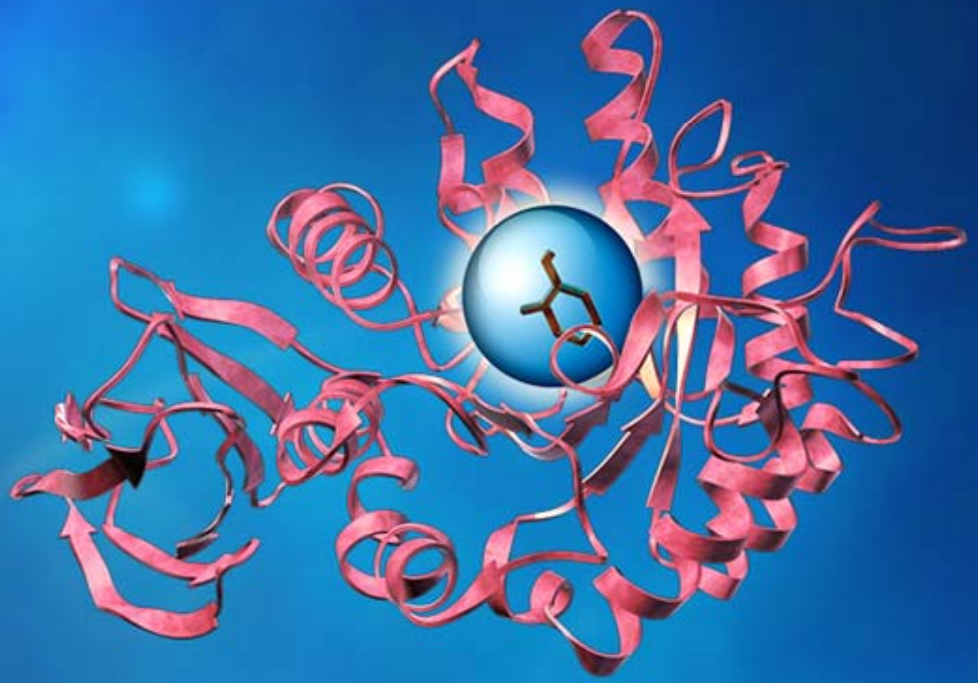
Amicus Therapeutics



2014 Leerink Global Healthcare Conference

William D. "Chip" Baird, Chief Financial Officer

Bradley L. Campbell, Chief Operating Officer

at the forefront of therapies for rare and orphan diseases

Safe Harbor

This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to business, operations and financial conditions of Amicus including but not limited to preclinical and clinical development of Amicus' candidate drug products, cash runway, ongoing collaborations and the timing and reporting of results from clinical trials evaluating Amicus' candidate drug products. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "plan," "would," "should" and "could," and similar expressions or words, identify forward-looking statements. Although Amicus believes the expectations reflected in such forward-looking statements are based upon reasonable assumptions, there can be no assurance that its expectations will be realized. Actual results could differ materially from those projected in Amicus' forward-looking statements due to numerous known and unknown risks and uncertainties, including the "Risk Factors" described in our Annual Report on Form 10-K for the year ended December 31, 2012. All forward-looking statements are qualified in their entirety by this cautionary statement, and Amicus undertakes no obligation to revise or update this presentation to reflect events or circumstances after the date hereof.

2014 Highlights

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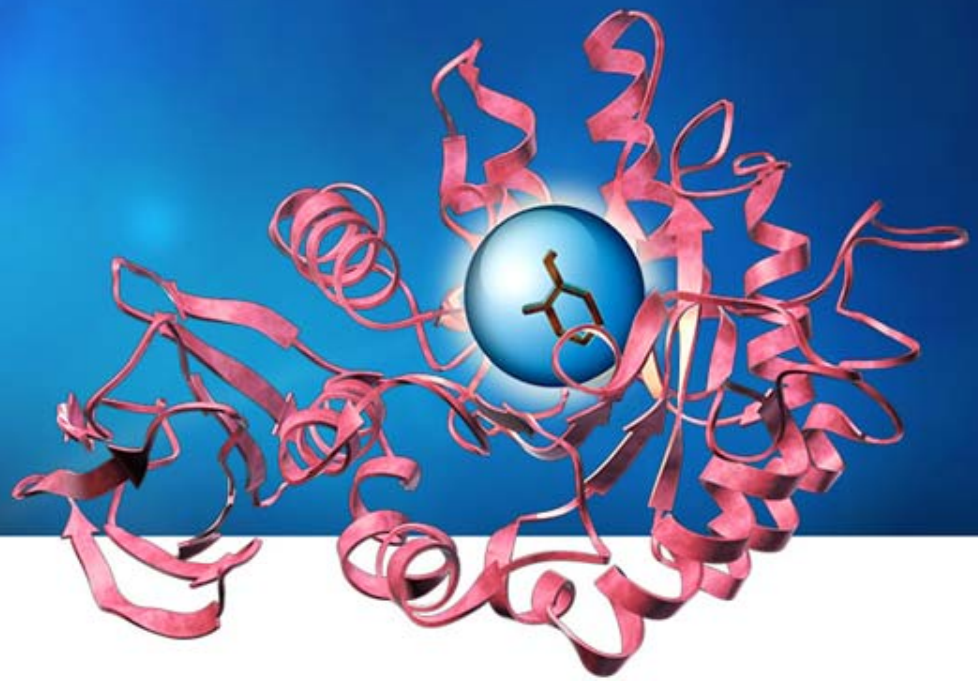

Lysosomal Disease Network WORLD Symposium Highlights

February 11-14, 2014

LDN WORLD 2014: Summary of Presentations and Posters

Oral Platform Presentations:

- **Pompe Disease**:

  Chemical Conjugation of Targeting Peptide to ERTs Improve Receptor Binding and Substrate Clearance in Mouse Models of Disease – Hung Do, PhD, Amicus Therapeutics, Inc. (Wednesday, February 12, 2014 at 2:45 p.m. PT)

- **Fabry Disease**:

  Phase 3 Study (FACETS) of Migalastat HCl for Fabry Disease: Post hoc GLA Mutation-Based Identification of Subjects Likely to Show a Drug Effect – Jeffrey P. Castelli, PhD, Amicus Therapeutics, Inc. (Thursday, February 13, 2014 at 11:15 a.m. PT)

- **Gaucher Disease**:

  Glucosylceramide and Glucosylsphingosine Quantitation by Liquid Chromatography-Tandem Mass Spectrometry to Enable Studies of Neuronopathic Gaucher Disease – Rick Hamler, Amicus Therapeutics, Inc. (Tuesday, February 11, 2014 at 10:30 a.m. PT)

Posters: Tuesday, February 11 - Thursday, February 13, 2014, 4:00-6:00 p.m. PT

- **Pompe Disease**:

  Chemical Conjugation of Targeting Peptide to ERTs Improve Receptor Binding and Substrate Clearance in Mouse Models of Disease

  Subcutaneous Administration of Recombinant Human Acid Alpha-Glucosidase Co-formulated with the Pharmacological Chaperone AT2220 Leads to Lysosomal Uptake of rhGAA and Glycogen Reduction in Disease-relevant Tissues of Pompe Mice

  Assessment of the Effect of Duvoglustat Co-administered with Acid Alglucosidase Alfa Infusion on the Immune Response to Enzyme Replacement Therapy in Pompe Disease

  Liquid Chromatography-Tandem Mass Spectrometry Determination of AT2220 in Rodent Plasma and Tissues

- **Fabry Disease**:

  Phase 3 Study (FACETS) of Migalastat HCl for Fabry Disease: Post hoc GLA Mutation-Based Identification of Subjects Likely to Show a Drug Effect

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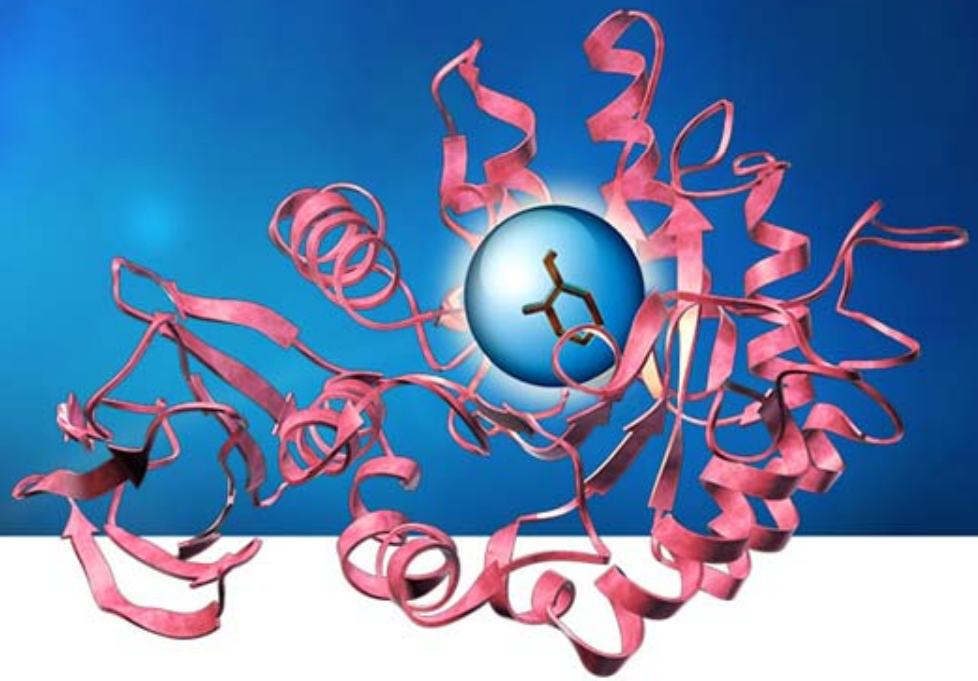


Next-Generation ERT for Pompe Disease

Three Challenges with Pompe ERT

Activity/ Stability

Rapid denaturation of ERT in pH of blood[1]

Uptake/ Targeting

Low M6P receptor uptake into skeletal muscle[2]

Majority of rhGAA is not delivered to lysosomes[2]

Tolerability / Immunogenicity

Infusion-associated reactions in ~50% of late-onset patients[3]

High antibody titers shown to affect treatment outcomes[4,5]

[1]Khanna *et al.,* **PLoS ONE**, 2012; [2]Zhu et al., Amer. Soc. Gene Therapy, 2009 June; [3]Banati *et al.,* **Muscle Nerve**, 2011 Dec. ; [4]Banugaria *et al.*, **Gen. Med.,** 2011 Aug.; [5]de Vries *et al.,* **Mol Genet Metab**., 2010 Dec.

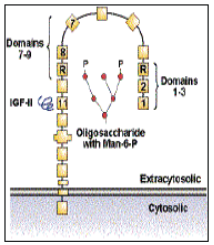
Complementary Technologies to Address ERT Challenges in Pompe Disease

Callidus Acquisition Provided Pompe ERT (AT-B200) and Platform Technology Complementary to Amicus' CHART™ Platform

CHART™
CHAPERONE-ADVANCED REPLACEMENT THERAPY

- Binds to and stabilizes rhGAA
- Increases uptake of active enzyme into tissues
- Improves tolerability and potentially mitigates immunogenicity

Callidus Technology



- Enzyme uniquely engineered with high M6P content and optimized carbohydrate structures
- Peptide tag (variant of IGF-2, or vIGF-2) further enhances drug targeting and uptake

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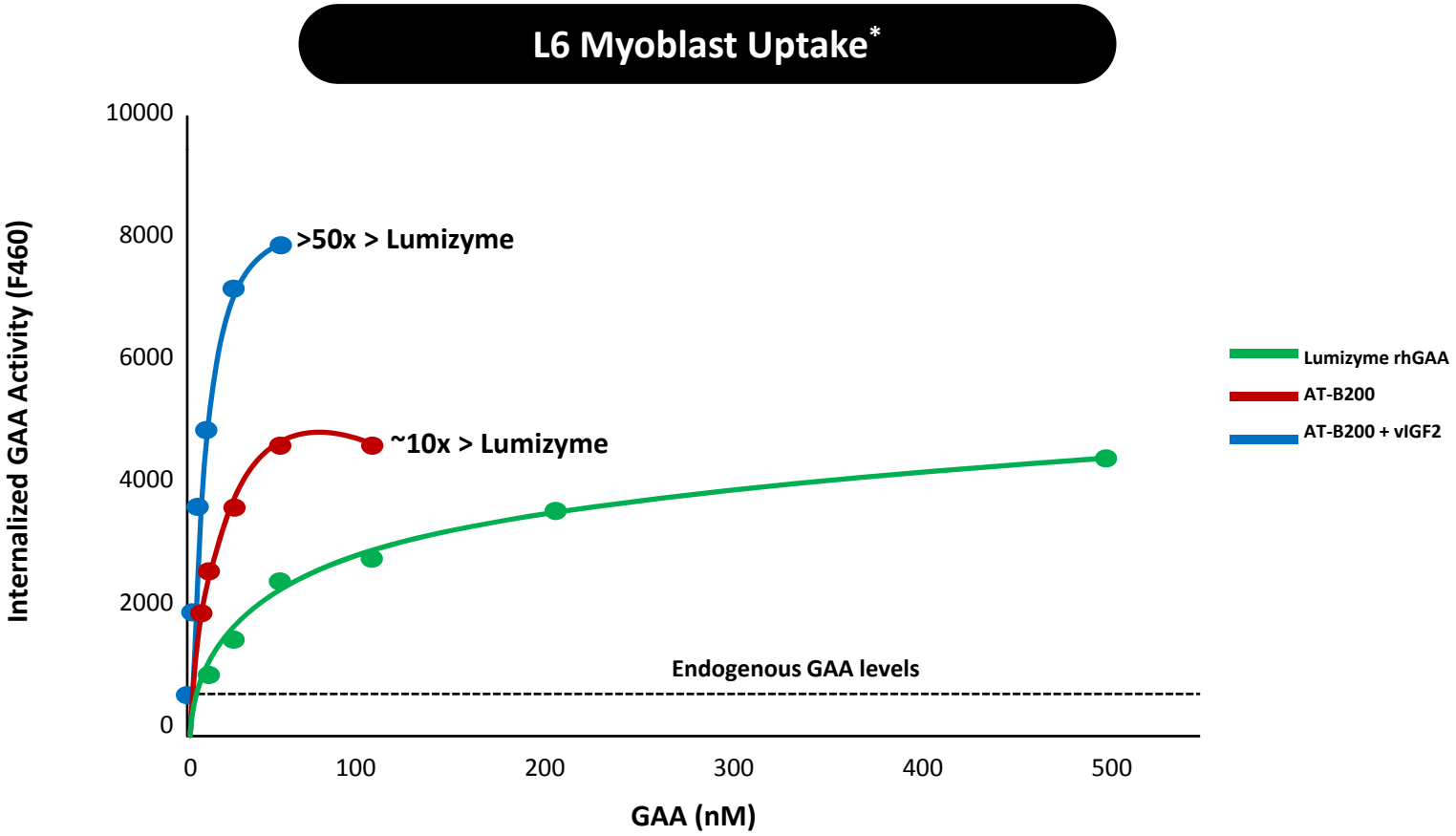
(Preliminary Results)

AT-B200: Next-Generation Pompe ERT (rhGAA)

AT-B200 Has Demonstrated Significant Advantages in Preclinical Studies that May Be Further Improved By Co-Formulating with a Chaperone



*Do *et. al*, LDN WORLD 2014

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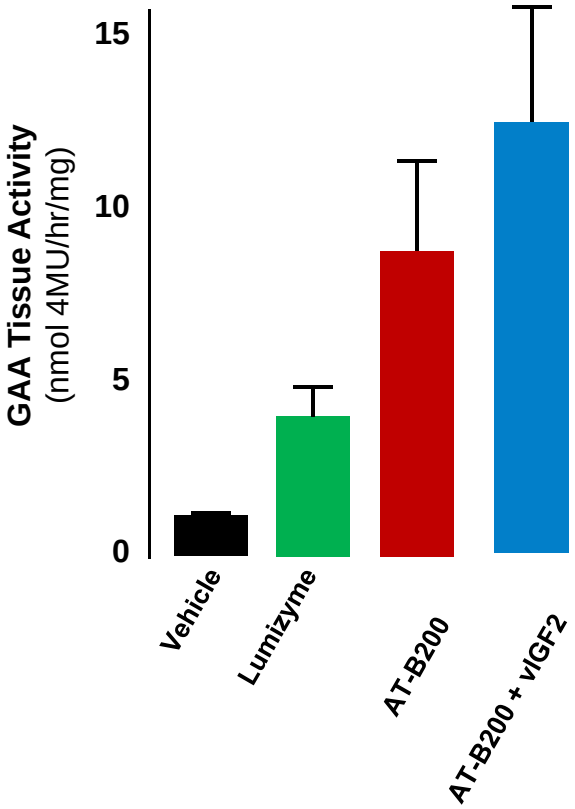
(Preliminary Results)

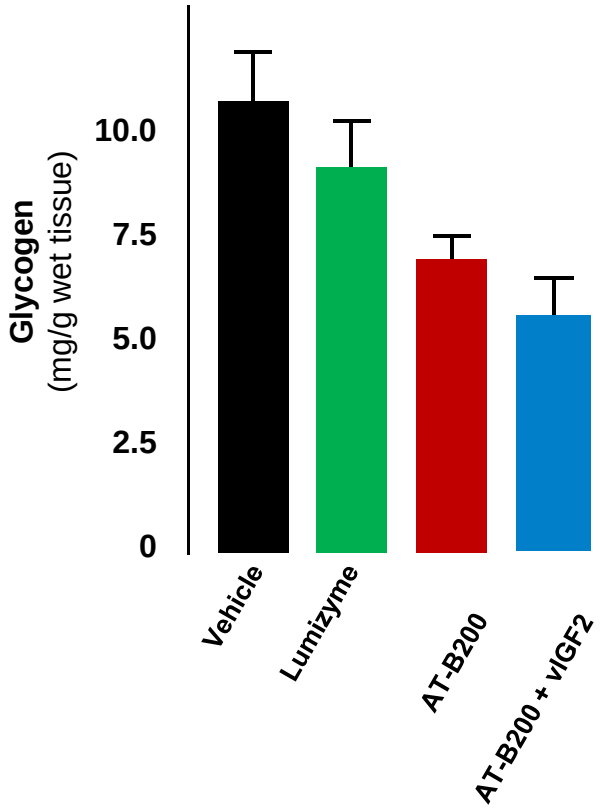
AT-B200: Next-Generation Pompe ERT (rhGAA)

AT-B200 Led to Better Uptake into Muscle and Further Glycogen Reduction Compared to Lumizyme in Preclinical Studies in GAA Knock-Out Mice*

GAA Activity in Quadriceps



Glycogen in Quadriceps



*Do *et. al,* LDN WORLD 2014; 4 weekly doses of each GAA preparation. Tissues harvested 1 week after last dose.

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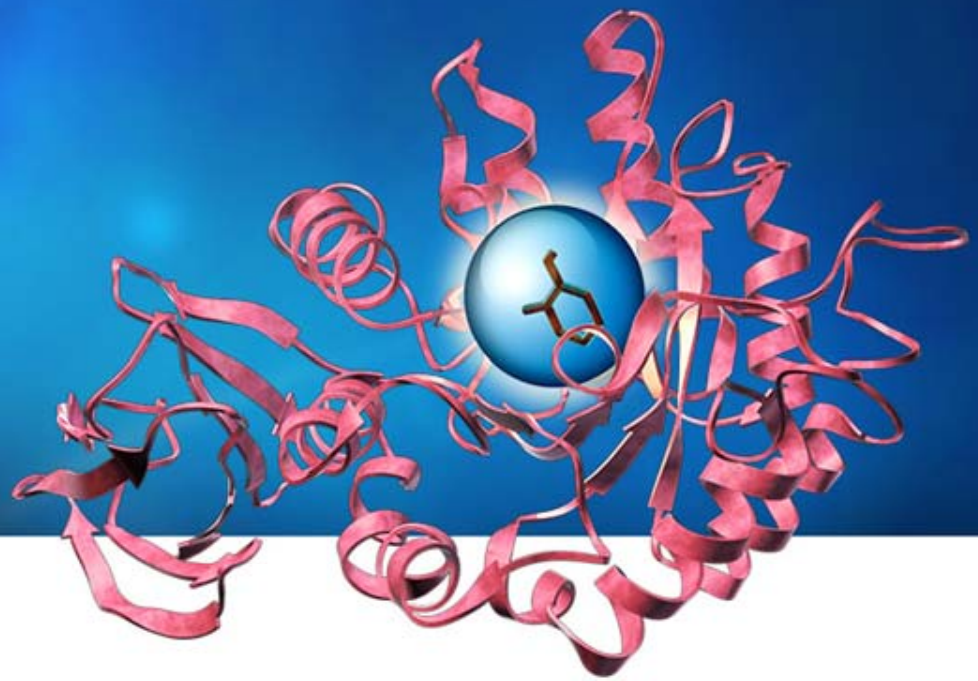
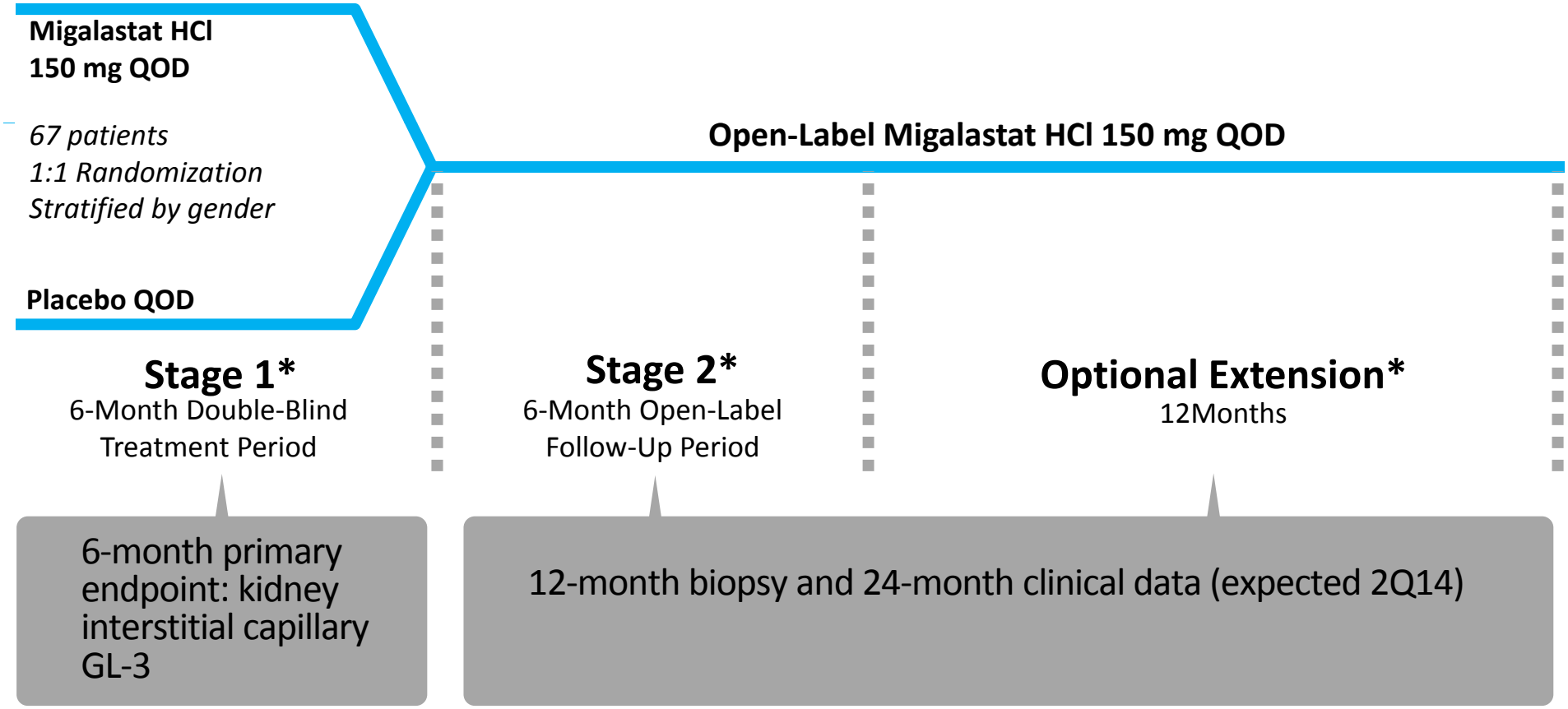


Chaperone Monotherapy for Fabry Disease

Phase 3 FACETS Study (Study 011)

Comparing Migalastat HCl 150 mg Every-Other-Day (QOD) to Placebo QOD in Stage 1, Followed by Open Label Migalastat HCl in Stage 2 and Optional Extension Phase



*GL-3 Substrate Measured by Histology in Kidney Biopsies **Clinical Outcomes Assessed, Including eGFR and Proteinuria

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Phase 3 Study 011: 6-Month Safety

No Serious Adverse Events Deemed by Investigators to be Treatment-Related
No Withdrawals Due to Adverse Events

Most Common Treatment Emergent Adverse Events (≥ 10% of Subjects)

Adverse event	Placebo (n=33)	Migalastat HCl (n=34)
Any Event	91%	91%
Headache	21%	35%
Fatigue	12%	12%
Nausea	9%	12%
Nasopharyngitis	6%	15%
Paresthesia	12%	9%

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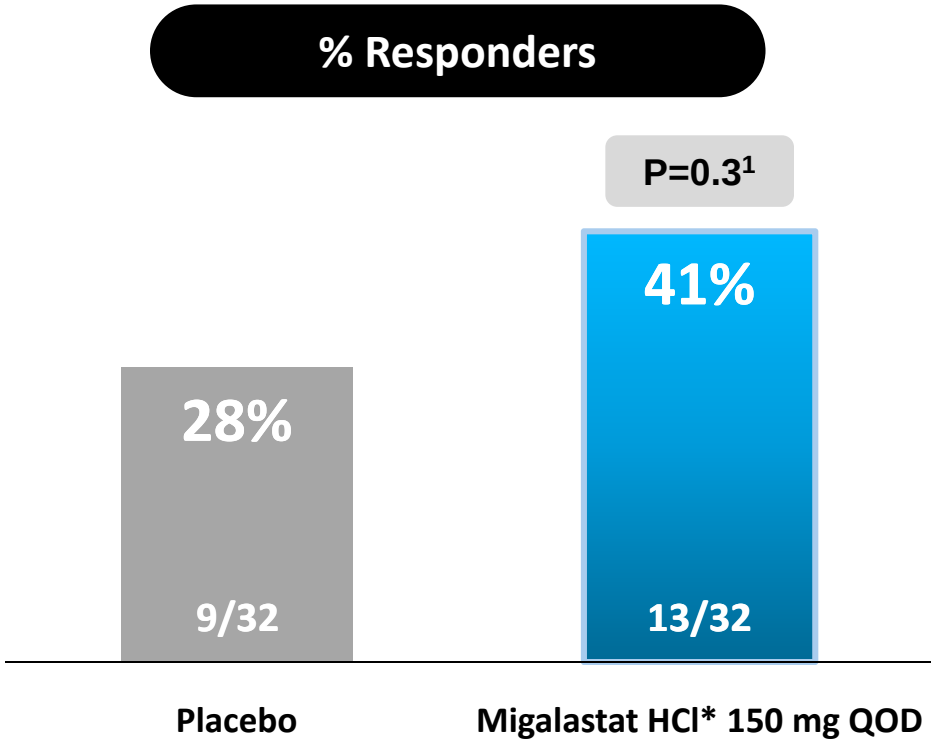
Phase 3 Study 011: Top-Line Stage 1 (6-Month) Results

Primary Endpoint - Responder Analysis (ITT): ≥ 50% Reduction from Baseline in Kidney Interstitial Capillary GL-3



* migalastat is not authorized for use and is an investigational product

1 Difference=12.5% (95% CI: -13.4, 37.3). Migalastat HCl minus placebo in % responders. P-value based on exact Cochran-Mantel-Haenszel test stratified by gender. Subjects with baseline biopsy but missing month 6 biopsy counted as a failure.

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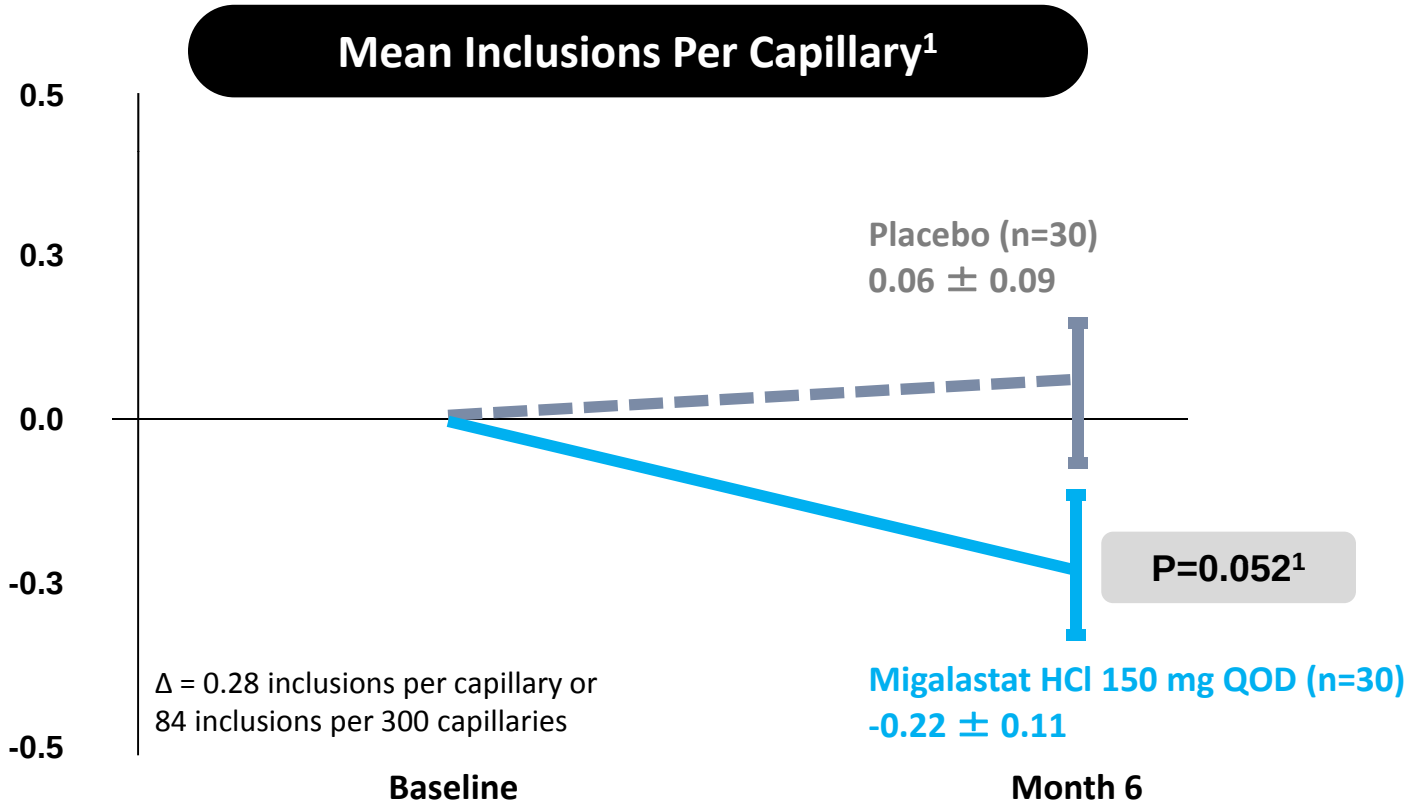
Phase 3 Study 011: Revised Statistical Analysis Plan

Post Hoc Kidney Interstitial Capillary GL-3 Analysis: Mean Change From Baseline Analyzed as Continuous Variable (Stage 1 mITT*)



*All patients with evaluable paired biopsies (baseline and month 6); n=30 per group. [1]Data points represent mean ± standard error (SEM) change from baseline in the mean number of GL-3 inclusions per capillary after 6 months of treatment with migalastat or placebo. Data analyzed using analysis of covariance (ANCOVA) model with covariate adjustment for baseline value and factors for treatment group and treatment by baseline interaction. P-value corresponding to least-square mean difference between migalastat and placebo is displayed.

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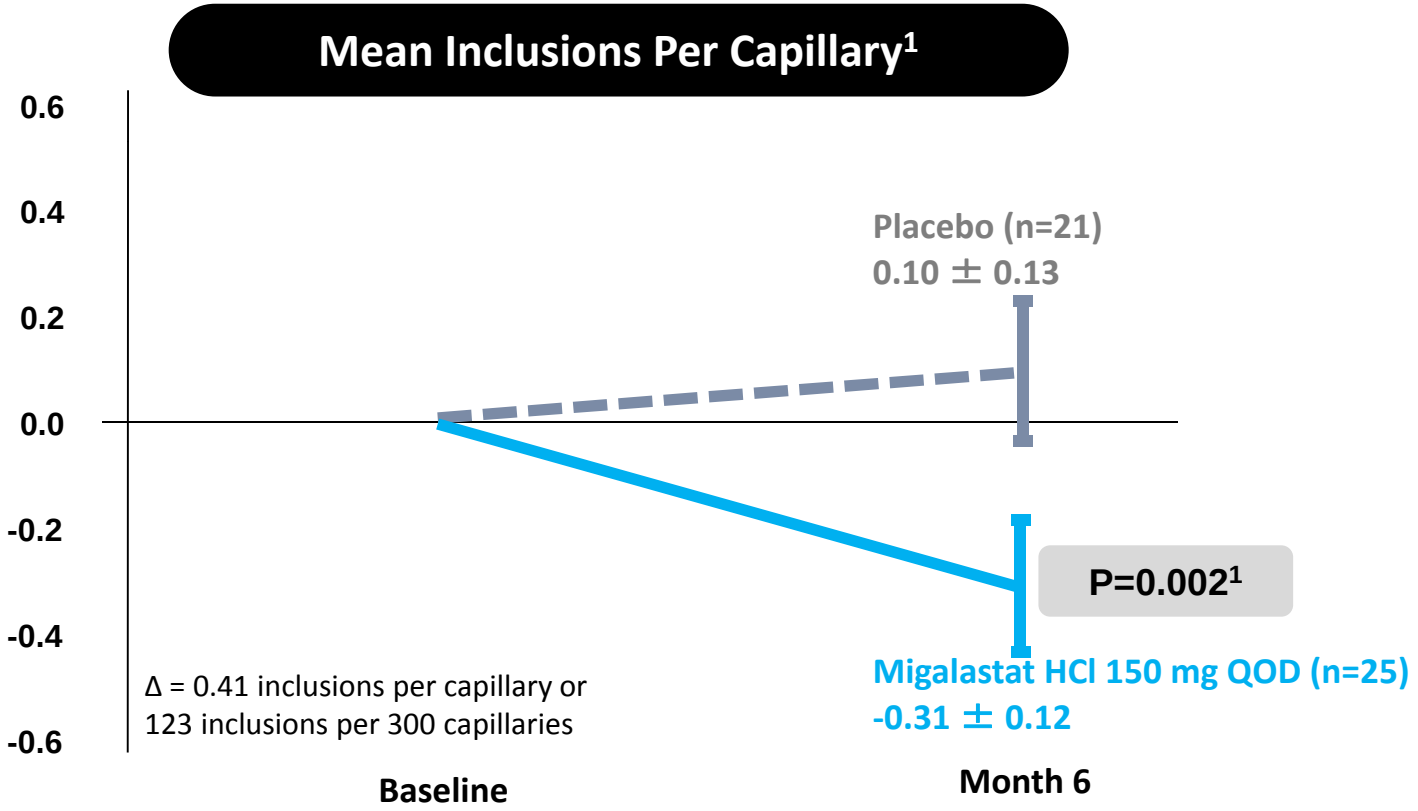
Phase 3 Study 011: Revised Statistical Analysis Plan

Post Hoc Kidney Interstitial Capillary GL-3 Analysis: Mean Change From Baseline Analyzed as Continuous Variable (GLP HEK Amenable*)



*All patients with evaluable paired biopsies (baseline and month 6) and amenable GLA mutations in GLP-validated HEK assay [1]Data points represent mean ± standard error (SEM) change from baseline in the mean number of GL-3 inclusions per capillary after 6 months of treatment with migalastat or placebo. Data analyzed using analysis of covariance (ANCOVA) model with covariate adjustment for baseline value and factors for treatment group and treatment by baseline interaction. P-value corresponding to least-square mean difference between migalastat and placebo is displayed.

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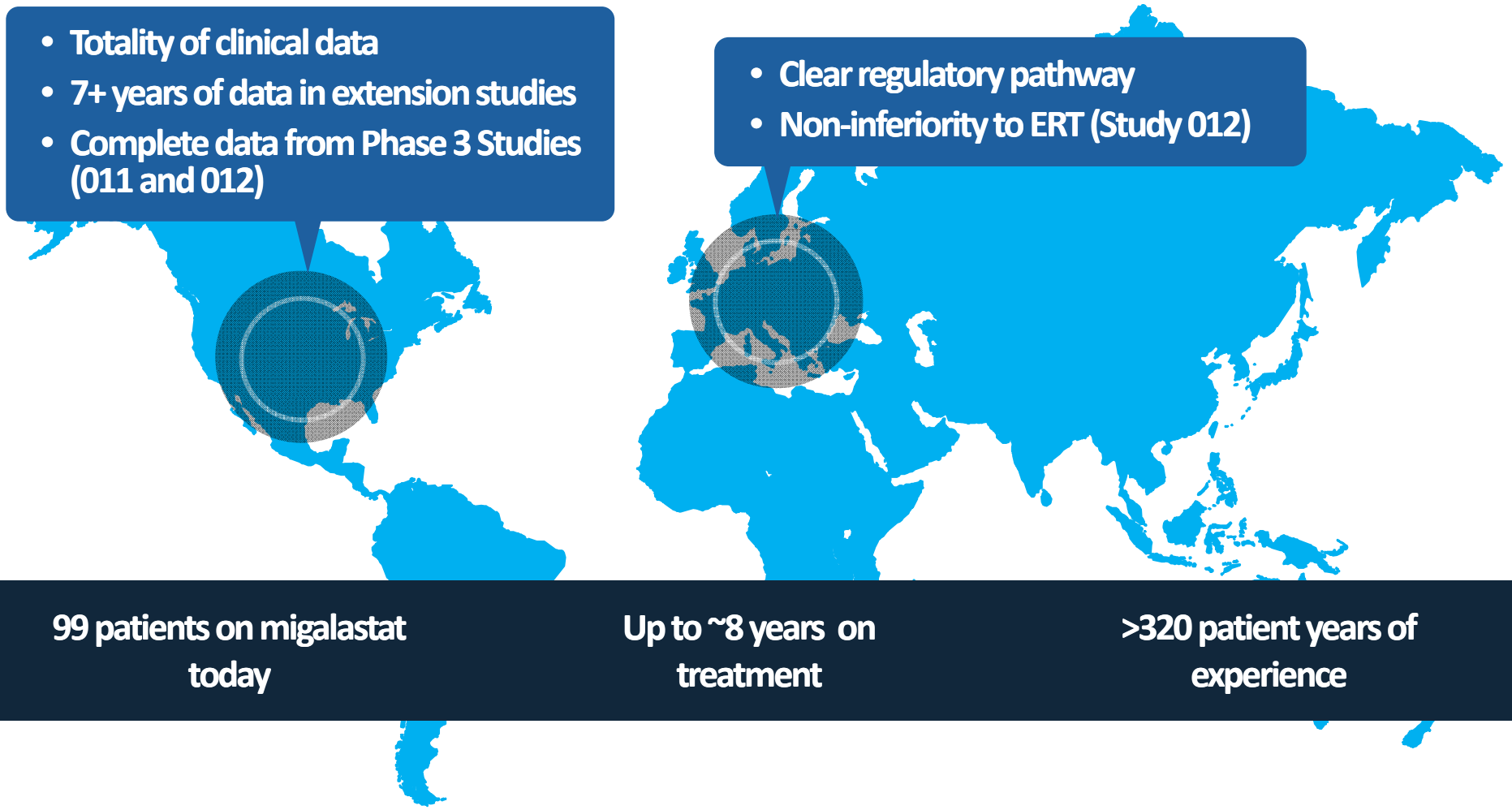
Global Regulatory Strategy

- Totality of clinical data
- 7+ years of data in extension studies
- Complete data from Phase 3 Studies (011 and 012)

- Clear regulatory pathway
- Non-inferiority to ERT (Study 012)



99 patients on migalastat today

Up to ~8 years on treatment

>320 patient years of experience

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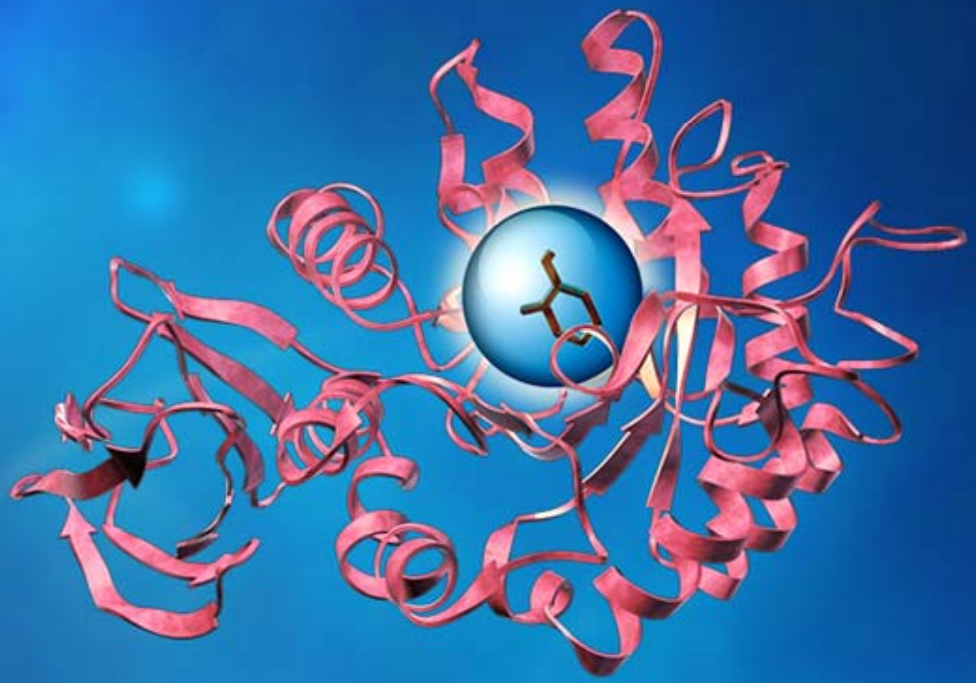

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