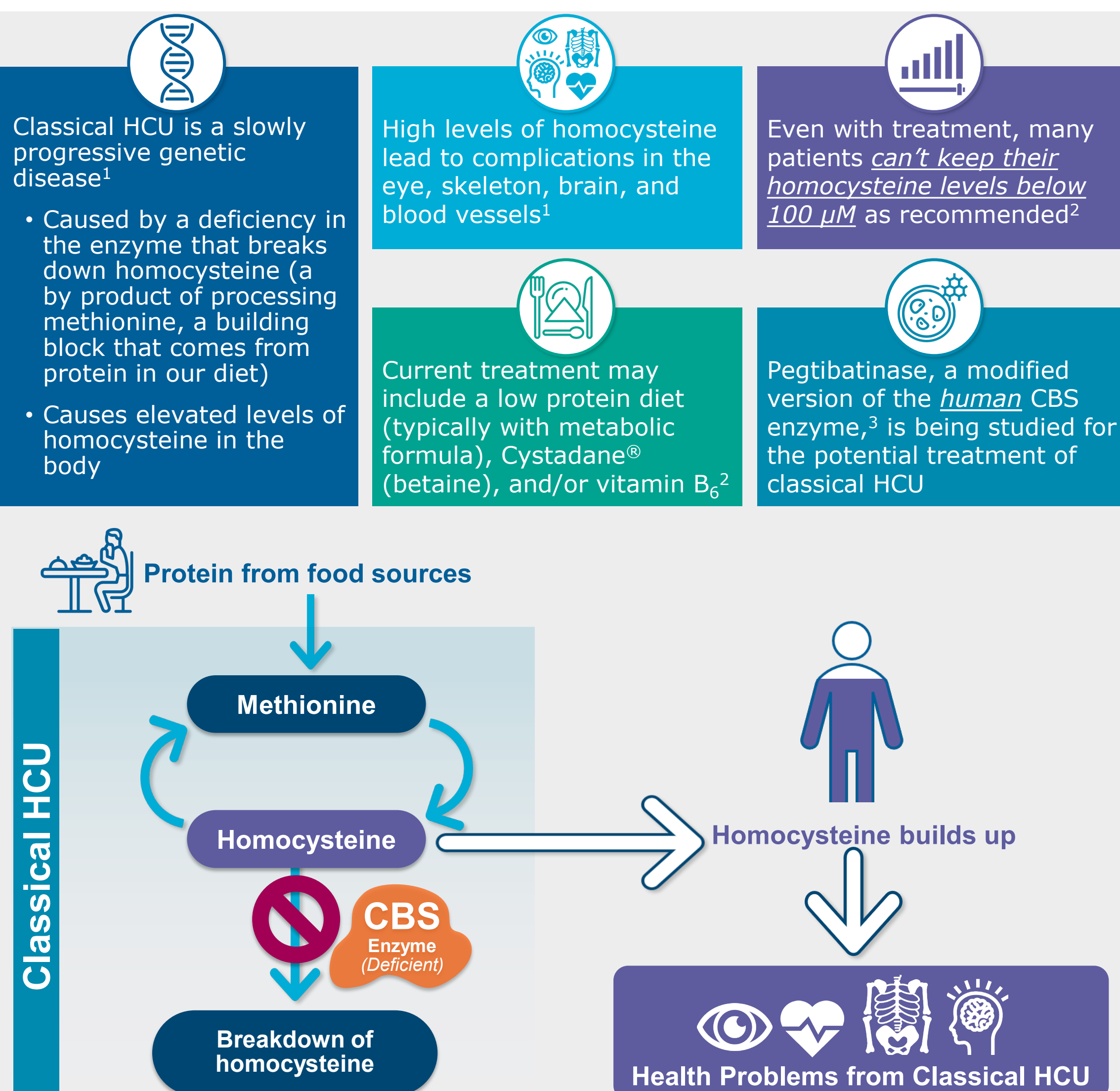


Pegtibatinase, an Investigational Enzyme Replacement Therapy for the Treatment of Classical Homocystinuria: Initial Results from the Phase 1/2 COMPOSE Study

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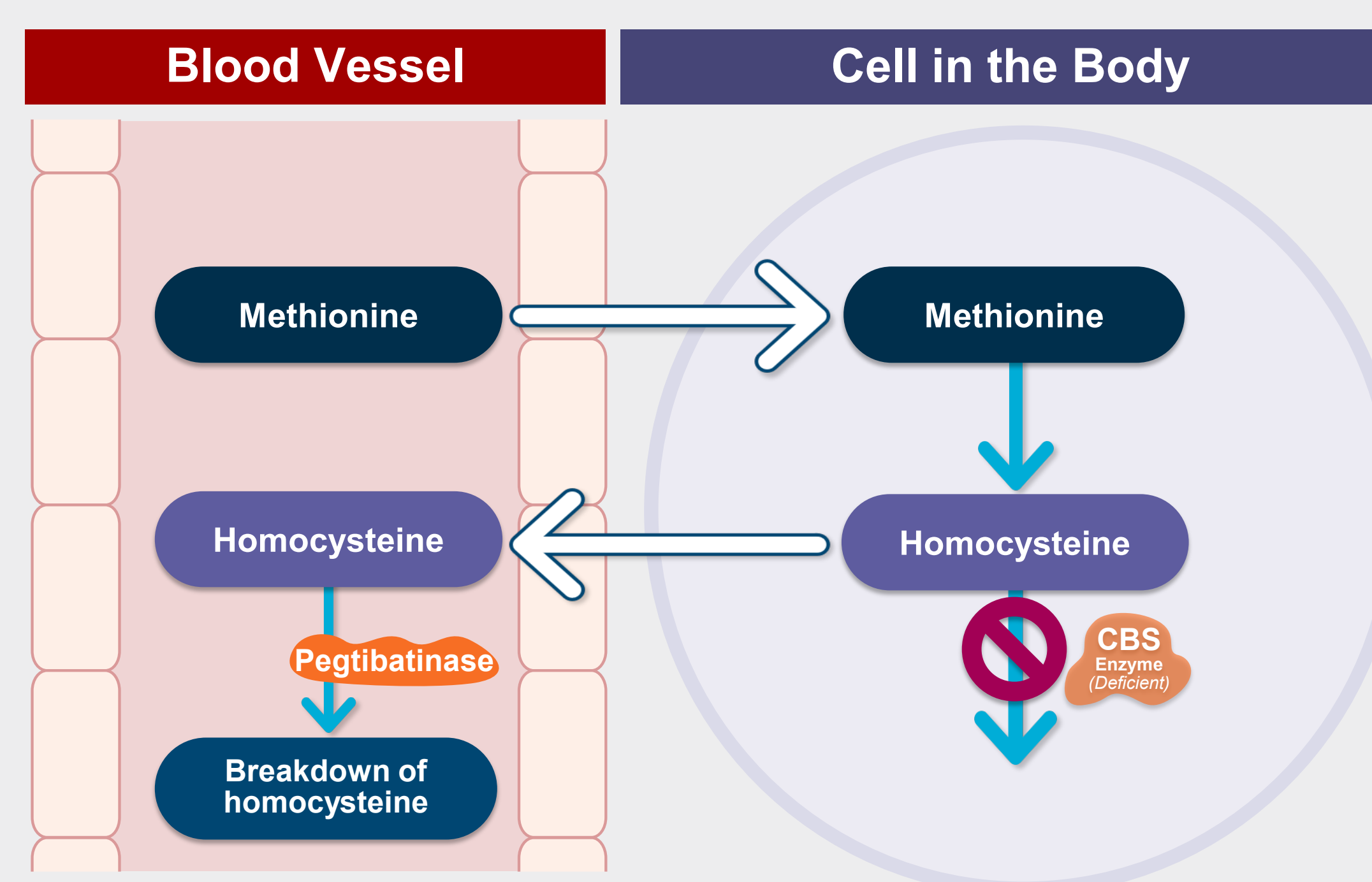
BACKGROUND



HYPOTHESIS

How Does Pegtibatinase Work? The Metabolic Sink Hypothesis⁴

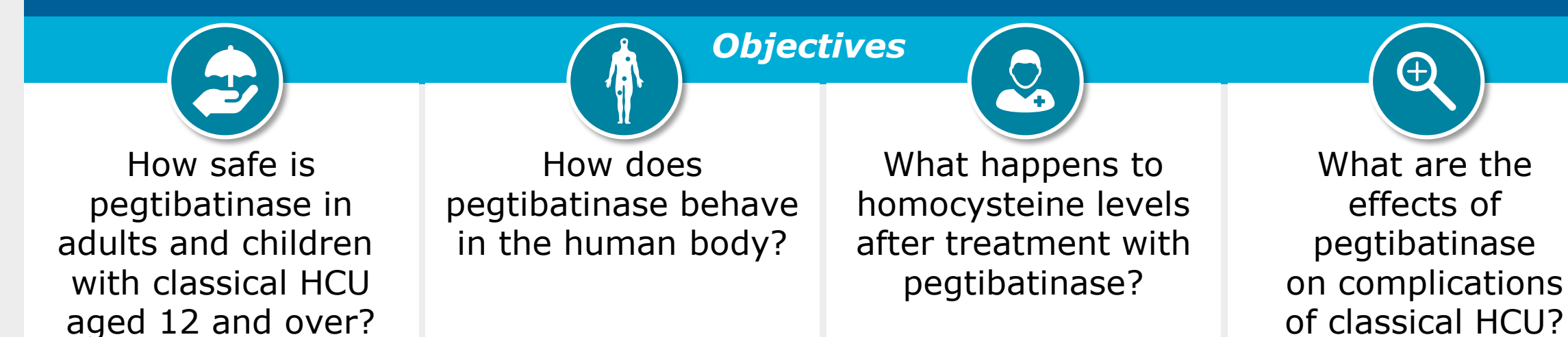
- We hypothesize that pegtibatinase can act as a "metabolic sink" and reduce levels of homocysteine and methionine in the blood and cells of the body⁴



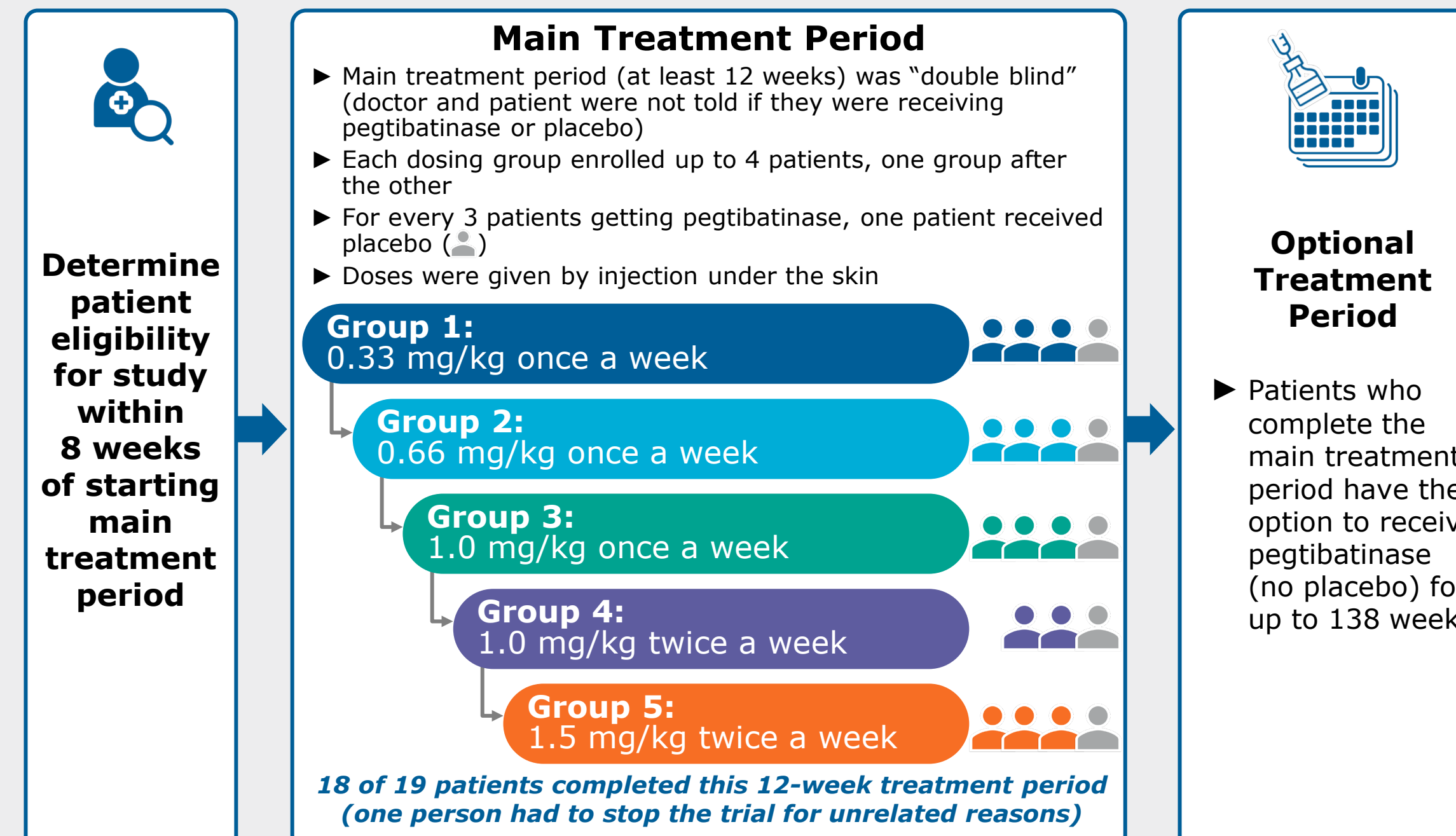
METHODS

COMPOSE is a clinical trial (first study in humans) to evaluate the safety and drug effects of pegtibatinase in patients with classical HCU

Compose

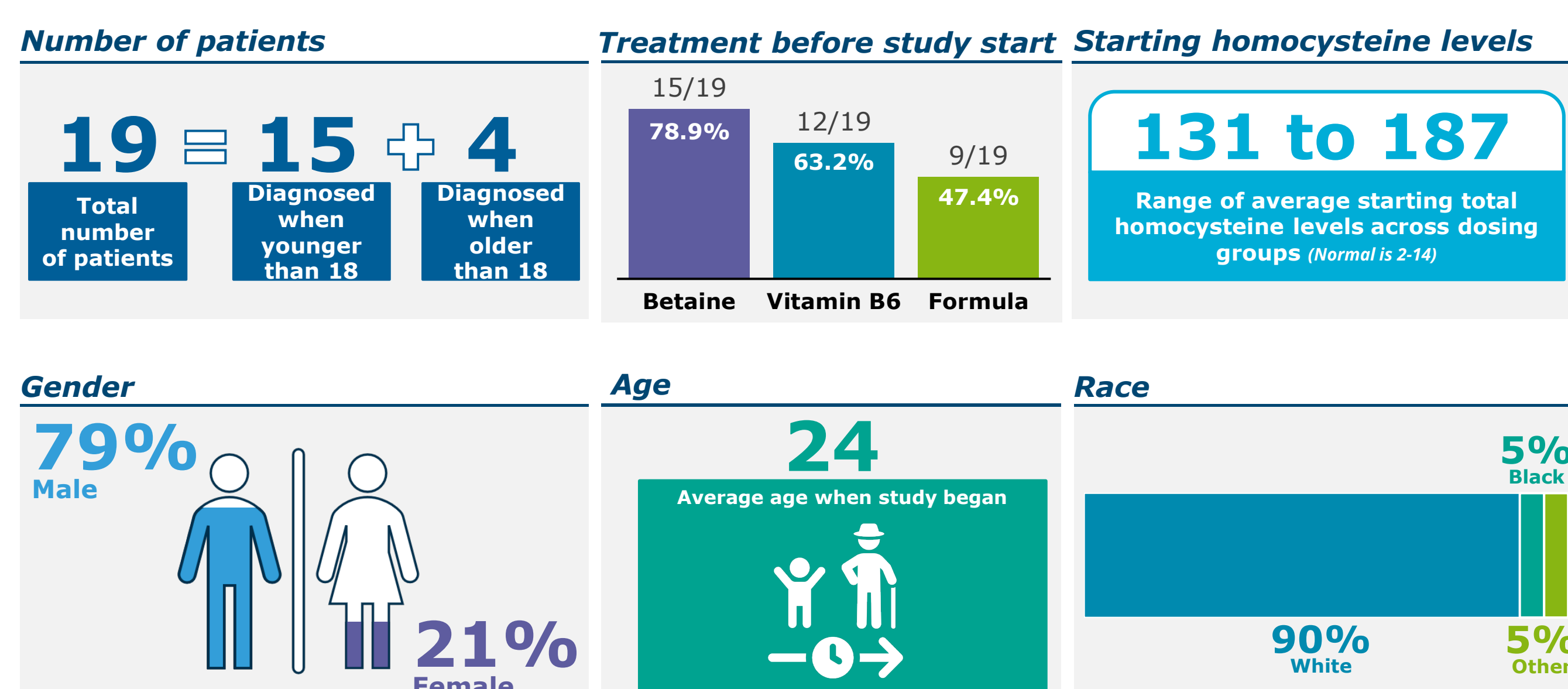


Study Design

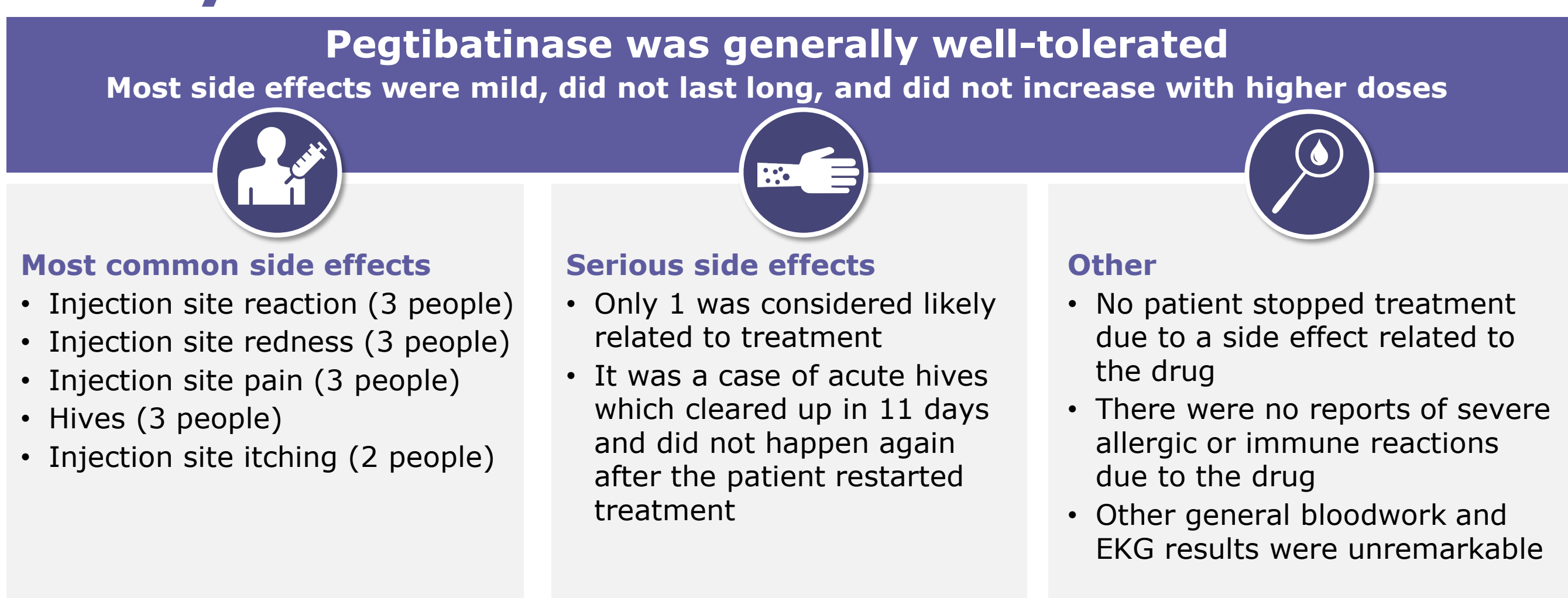


RESULTS

Patient Population^{4,5}

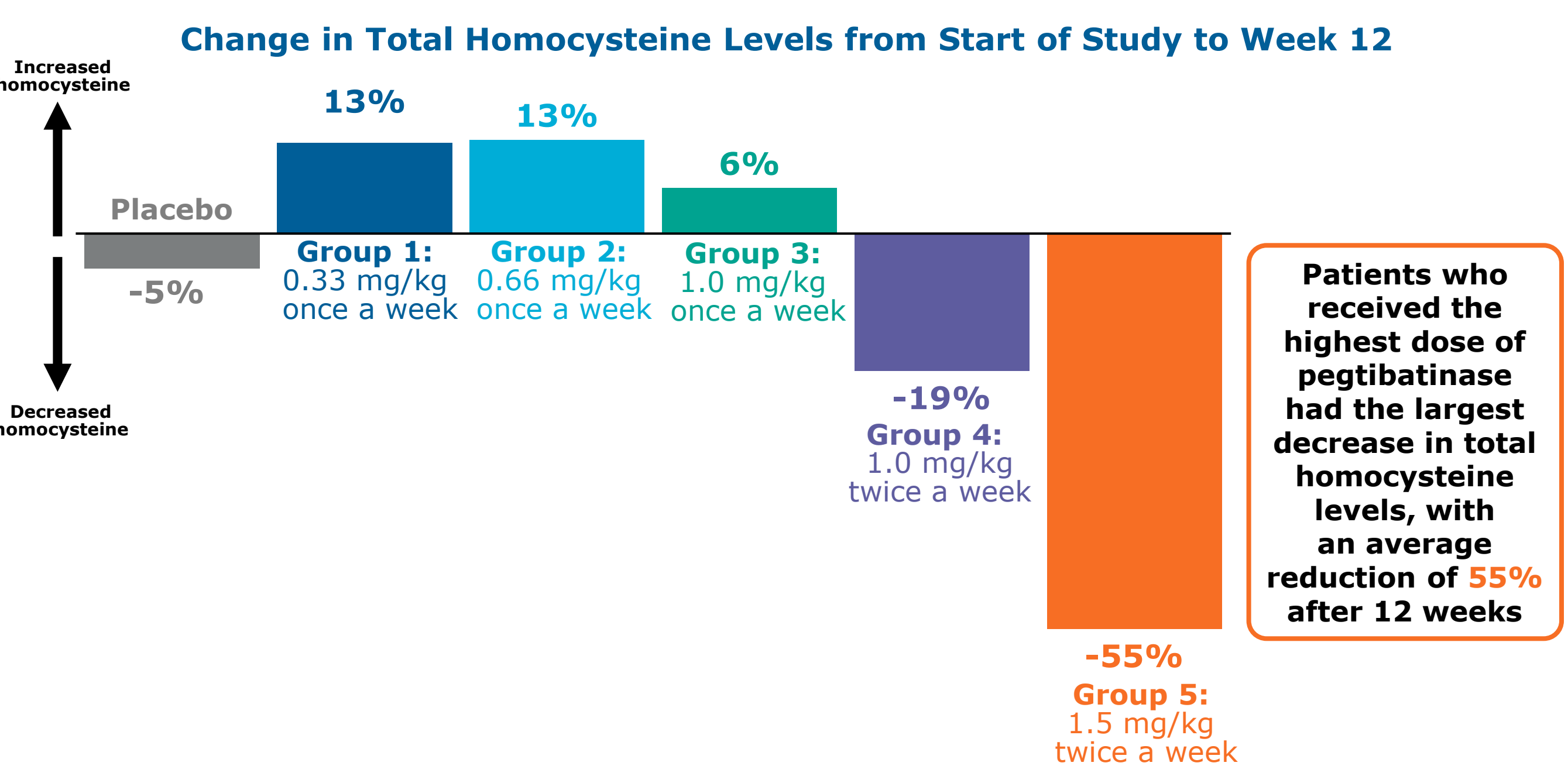


Safety



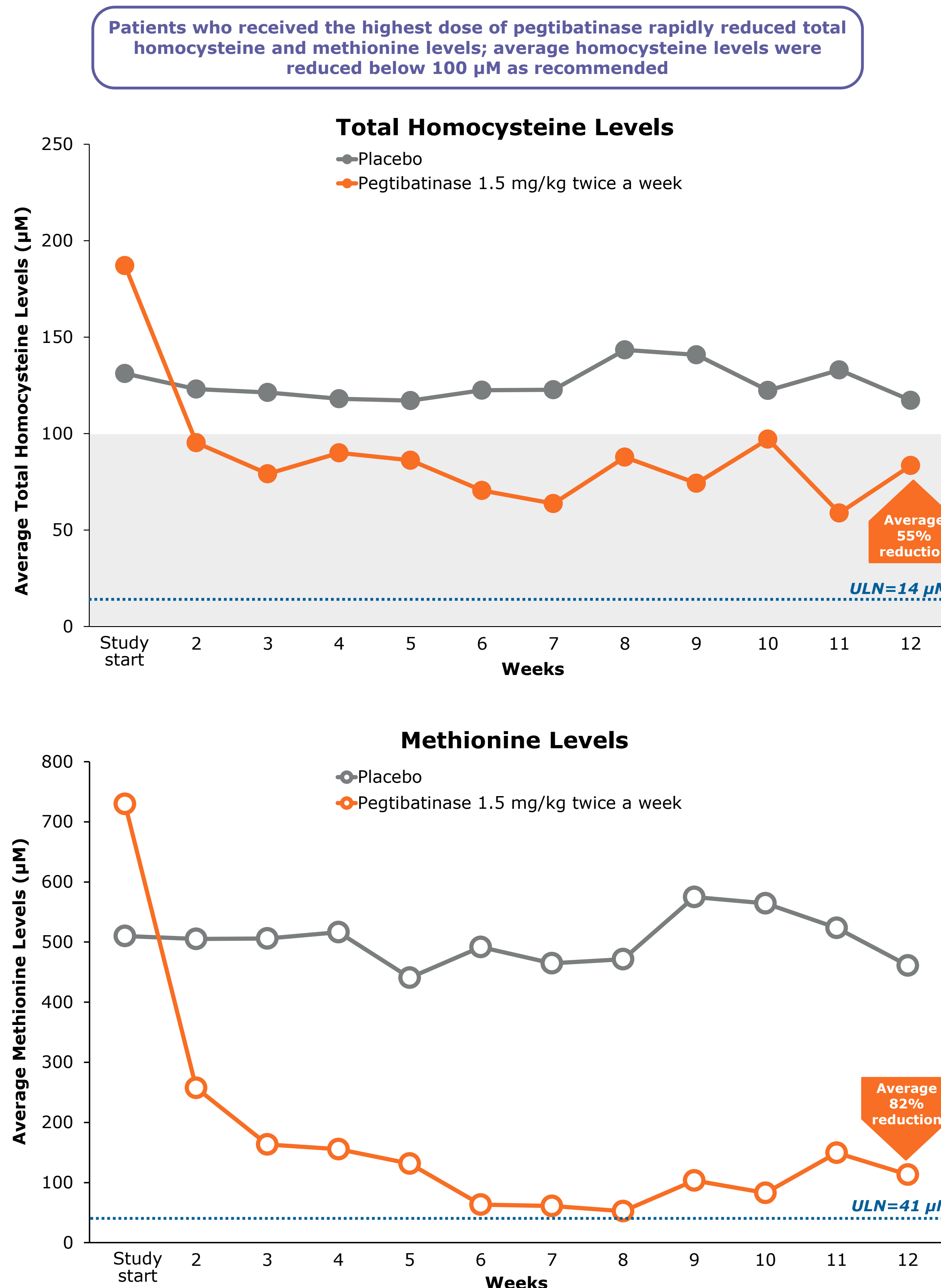
Efficacy

How Pegtibatinase Affected Homocysteine Levels



Efficacy (continued)

How Pegtibatinase Affected Homocysteine and Methionine Levels Over the 12 Weeks of Double-blind Treatment of the Study



CONCLUSIONS

- Pegtibatinase was generally well tolerated at all doses tested and there were no reports of severe allergic or immune reactions due to study drug; no patients stopped treatment due to side effects
- Patient groups on higher doses of pegtibatinase showed rapid reduction in total homocysteine levels; patients treated with the highest dose had an average reduction of 55% at 12 weeks
- Patients treated with the highest dose of pegtibatinase twice weekly had a sustained reduction of homocysteine over 12 weeks and maintained their average homocysteine level below 100 µM as recommended
- These results suggest that pegtibatinase may have the potential to be a new treatment for HCU

DISCLOSURES

HL: Investigator, Traverse Therapeutics, Inc.; consultant, Traverse Therapeutics, Inc. JT, CF, ML, JG: Investigator, Traverse Therapeutics, Inc. MS-M: Consultant, Traverse Therapeutics, Inc. EMB, LW, JG, YC, FG, MS, SAV: Employees and stockholders, Traverse Therapeutics, Inc.

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