Alpha$_1$-proteinase inhibitor

Product:
ALPHA$_1$-PROTEINASE INHIBITOR (Prolastin®) injection

Class of drugs:
Alpha$_1$-antitrypsin replacement

Indication:
Alpha$_1$-antitrypsin (AAT) deficiency

Manufacturer:
Talecris Biotherapeutics Inc.

CED Recommendation

The CED recommended that alpha$_1$-proteinase inhibitor (Prolastin®) not be funded on the basis that this drug has not been shown to provide meaningful clinical benefit.

Executive Officer Decision

Based on the CED's recommendation, the Executive Officer decided not to fund alpha$_1$-proteinase inhibitor (Prolastin®) through the Ontario Public Drug Programs.

Status

Not funded by the Ontario Public Drug Programs.

Highlights of Recommendation:

- Prolastin is an injectable drug marketed for the treatment of emphysema caused by alpha1-antitrypsin (AAT) deficiency.
- Randomized controlled studies showed that Prolastin therapy did not improve lung function, rate of emphysema exacerbation, or quality of life.
- There have been no clinical studies to assess whether Prolastin improves important health outcomes such as survival and the need for lung transplant.
- There are some observational data to suggest that patients treated with Prolastin show a slower decline in lung function. However, observational data are less rigorous and may not reliably ascertain treatment benefits.
- Prolastin costs approximately $90,000 per patient per year. Its cost cannot be justified given the lack of evidence of clinical efficacy.
- **Overall, the CED recommended that Prolastin not be funded because therapeutic effectiveness and value for money have not been demonstrated.**

Background:

Alpha$_1$-antitrypsin (AAT) deficiency is an inherited disorder in which the body does not make enough of the AAT protein. The AAT protein (also known as alpha$_1$-proteinase inhibitor) protects the lungs and liver from damage. A lack of AAT may lead to lung and liver diseases, such as emphysema and cirrhosis.

Prolastin is an AAT preparation made from human plasma of blood donors. Prolastin is marketed for the treatment of emphysema caused by AAT deficiency. It has not been shown to cure the disorder nor reverse any damage already caused to the lungs.

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Detailed Discussion:

- There are three small randomized controlled studies that compared Prolastin to placebo. The studies consistently showed that Prolastin did not improve the rate of decline in lung function, rates of emphysema exacerbation, or quality of life.
- There are no trials evaluating the effect of Prolastin on the need for lung transplants, disability and survival. In two of the three randomized controlled studies, lung density was evaluated as an attempt to measure disease progression. The studies reported that Prolastin did not have a significant effect on lung density, although a trend towards positive response with Prolastin was seen. It is important to note that there is no evidence to indicate that lung density correlates with emphysema exacerbation rates, the need for lung transplant, mortality or any other clinically significant health outcomes. Therefore, this trend has unknown clinical relevance.
- Some observational studies from patient registries reported that patients who received Prolastin showed a slower decline in lung function compared with those who did not receive treatment. However, it is unknown whether the difference in lung function observed was due to Prolastin or to other factors such baseline differences that were unequal between the patients.
- Prolastin costs approximately $90,000 per patient per year. There is a lack of evidence to demonstrate reasonable clinical efficacy with this drug to justify the treatment cost.
- **Overall, the CED recommended that Prolastin not be funded given that therapeutic effectiveness and value for money have not been established.**

Ontario

Ministry of
Health and Long-Term Care
Ontario Public Drug Programs

For more information, please contact:

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