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*Title* **Human Alpha1-Proteinase Inhibitor for Patients with Alpha1-Antitrypsin Deficiency**

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## Aim

To assess the clinical and cost effectiveness of α1-proteinase inhibitor (α1-PI) compared with no human α1-PI in patients with ATT (antitrypsin) deficiency, and to determine the status of public funding for α1-PI in Canada and other countries.

## Conclusions and results

In controlled trials, augmentation therapy has not shown reduced lung function impairment in patients with AAT deficiency and chronic obstructive pulmonary disease (COPD), compared with normal care. Conversely, in observational studies, α1-PI is associated with outcomes suggestive of therapeutic benefit in patients with severe AAT deficiency and moderate airflow obstruction. Severe adverse events from treatment have been reported in ~1% of study populations. No evidence was found evaluating the use of α1-PIs in patients with AAT deficiency and no lung function impairment.

Based on the only cost-utility analysis conducted, lifetime costs could average almost 1 million US dollars (USD) to produce 2.57 quality-adjusted life-years (QALYs) (from 4.62 with standard care to 7.19) resulting in a ratio of USD 333 349 per QALY. It is anticipated that Canadian costs would be similar. Public funding of α1-PI varies across Canada, and although the product is marketed in several European countries where funding is provided, the funding details are not known.

## Recommendations

Not applicable.

## Methods

We systematically reviewed the clinical and economic literature. Information regarding funding and delivery in Canada and similar public health systems was collected and synthesized.

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