Type: case study

Structure: problem statement, description, lessons learned, stakeholder perspective

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Topic: Health Policy & Regulatory

Subtopic: Reimbursement & Access Policy

Disease: Rare diseases

Title: Innovative Mechanisms of Pre-Market Authorization Access for Rare Diseases in Brazil: A case study of Pabinafusp-alfa for Mucopolysaccharidosis Type II

Problem statement: Hunter syndrome (HS) is a rare, genetic X-linked recessive lysosomal storage disease. Clinical manifestations vary widely in severity and involve multiple organs and tissues; central nervous system (CNS) symptoms are present in approximately two thirds of patients, being associated with poor prognosis and high burden.

Description: In Brazil, although guidelines cover diagnosis, supportive and enzyme replacement treatment (ERT) for HS without CNS manifestations, no specific treatment is available for patients with CNS symptoms. Pabinafusp-alfa crosses the blood-brain barrier, overcoming current challenges to treat the neuronopathic manifestations of HS and help maintain or improve cognitive function in patients. Currently pabinafusp-alfa is available only in Japan and Brazil; in the latter, it is only available nested to research protocols. Studies with pabinafusp-alfa started in Brazil in 2018 (Phase II) as result of direct and organized demand from rare diseases patient associations to drug manufacturer. Despite lack of healthcare authorities' involvement, up to 2022, 20 patients were treated in Brazil, resulting on estimate savings of USD 13 million for public healthcare system with ERT and providing better patient outcomes.

Lessons Learned: Due to patient organizations engagement, it was possible to provide access for an orphan drug before regulatory approval. Innovative access alternatives can provide faster access to treatments for patients with unmet needs, especially with orphan diseases. Other alternatives include a) access through clinical studies, with execution/development aligned with healthcare managers and linked with

potential future access strategies; b) risk sharing at regulatory level, considering the uncertainties in effectiveness and possibly market withdrawal and/or reimbursement to the system in case of negative results; c) pre-delivery, with subsequent payment only if the results of the clinical study are positive.

Stakeholder perspective: multiple, including patients/caregivers, providers, payers, and industry.