

## PP20 Health Technology Assessment To Sustain The Health Insurance Scheme: A Case Study On Anemia Treatment In Ghana

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**Introduction:** In Ghana, the reimbursement of iron polymaltose complex (IPC) for iron-deficiency anemia treatment raises concerns as a potential cost driver for the National Health Insurance Scheme (NHIS). Prioritizing value for money is crucial for NHIS sustainability. With Ghana's established health technology assessment (HTA) framework, we provide insights and preliminary findings in this case study on anemia treatment.

**Methods:** To further support institutionalization, we follow the Ghana HTA process guidelines and Ghana HTA reference case. Simultaneously, to build wider capacity for HTA in Ghana, relatively new trained staff was engaged while anchoring the team at the HTA Secretariat from the Ministry of Health. We started a situational analysis, which includes desktop review and key-stakeholder interviews. An umbrella review was commenced simultaneously to identify systematic reviews assessing clinical effectiveness of IPC compared to other medicines on the essential medicine lists for all population groups. We will assess cost-effectiveness to ultimately inform coverage decisions and possible implications for organizational structures.

**Results:** Anemia is a persistent public health issue in Ghana, impacting children under five, young women, and expectant mothers. The essential medicine list includes various treatments, but ambiguity exists in the standard treatment guidelines regarding when to administer IPC versus alternative medicines. Under the NHIS, the intravenous formulation of IPC stands out as the most expensive treatment. A systematic review identified three papers, focusing on infants, children, adults, and pregnant women. Preliminary findings suggest weak overall evidence supporting IPC's superiority, although adults may exhibit higher tolerance for IPC compared to alternative treatments.

**Conclusions:** Conducting an HTA on anemia treatments, despite the low treatment costs, is still significant as technical efficiencies can be achieved with high-volume drugs by analyzing prescription patterns and generating evidence to support potential changes. Findings from this HTA can be used elsewhere, especially in resource-constrained settings. Next steps involve finalizing the HTA and disseminating results to stakeholders.

## PP21 Examining Clinical Evidence: Incorporation Of Medications For Ultra-Rare Diseases – Descriptive Analysis Of Conitec Reports (2012 to 2022) – Brazil

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**Introduction:** The decision-making process for health technology assessment (HTA) in ultra-rare diseases faces a significant challenge for agencies worldwide. This study sought to offer an analytical overview of the clinical evidence outlined in the recommendations of the Brazilian National Committee for Health Technology Incorporation (Conitec) in ultra-rare diseases.

**Methods:** Data were extracted from recommendation reports for the ultra-rare diseases evaluated between 2012 and 2022. To classify a disease as ultra-rare, the epidemiological criterion or a consultation with the Orphanet platform was used (prevalence of  $\leq 1/50,000$  inhabitants). The extracted variables included the type of evidence synthesis, type of studies, instrument, the result of the assessment of the methodological quality of the studies, the format of evidence synthesis presentation, whether the evidence was graded, and the result.

**Results:** Among 53 analyzed reports, 70 percent relied on randomized controlled trials, followed by systematic reviews (SR), and observational studies. Reports with positive recommendations based on SR comprised 63 percent. GRADE applied to 27 reports and indicated low or very low results for the first two outcomes (62% and 65%). No clear link between evidence quality and final recommendations was observed. Meta-analysis-based reports had 83 percent positive recommendation rate, compared to 55 percent without meta-analysis. Surrogate outcomes were predominant. Clinical characteristics significantly influenced final decisions, especially when new data emerged in public consultation or had the potential to alter disease progression, reduce severe events, or enhance survival.

**Conclusions:** Ultra-rare diseases pose challenges in evidence quantity and quality. Traditional HTA frameworks seem inadequate, lacking robust evidence for these conditions. The difficulties in ultra-rare disease HTA underscore the need for specialized frameworks. This analysis acknowledges limitations, notably the heterogeneity in older report structures compared to recent ones, reflecting evolving HTA methodologies in Brazil.