

pilots have identified other areas that could benefit from further refinement, for example the active engagement of patient group representatives and clinical experts, rules and principles related to the handling of confidential information.

**CONCLUSIONS:**

Based on the limited number of REA pilots for medicines it is too early to draw final conclusions on the state of EU-level collaboration. But first signals indicate a positive development compared to REA pilots conducted in JA2. Interim evaluations are recommended to assess progress, and capture learnings for future pilots.

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## OP164 Hospital Budget Impact Of High-Cost Drugs: The Case Of Nusinersen

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**INTRODUCTION:**

Nusinersen is an orphan drug for spinal muscular atrophy (SMA) recently approved for marketing. Its high cost, striking but limited evidence of efficacy, and strong demand by media and patient organizations have generated a health policy conflict. We analyze the flaws of available evidence on nusinersen and its budget impact at a pediatric hospital, and report a collaborative strategy for drug procurement and financing.

**METHODS:**

Nusinersen is the highest-cost drug assessed by our hospital-based health technology assessment (HB-HTA) program so far. At the time of our assessment, only interim-analysis data of the pivotal randomized trial submitted to Federal Drug Administration (FDA) for approval and the European Medicines Agency (EMA) report containing unpublished final results were available. These secondary sources and other published phase II results were appraised. As a referral hospital, we concentrate most of the 300 SMA patients in our country. Hospital budget impact estimation included drug and hospitalization costs for the first and following years. The HTA report was submitted to the Ministry of Health to address this financing issue.

**RESULTS:**

The available evidence of efficacy raised serious methodological and clinical uncertainties. First-year treatment cost per patient was estimated in ARS 13,008,688 (USD 752,000, 10 percent of pharmacy annual drug budget). Hospital budget impact (70 eligible patients) was ARS 910,608,160 (USD 52,000,000; 18 percent of total annual hospital budget). Our recommendation was to contact central level authorities to resolve both drug financing and patient access by negotiating a shared-risk approach for an expanded access program, allowing further data collection for reassessment after 12 months. This, in turn, fostered mutual collaboration and consensus within the health system where several lawsuits were demanding drug coverage. Negotiation with the industry was initiated by the Ministry.

**CONCLUSIONS:**

This case is a clear example of forthcoming ultra-high-cost drugs unaffordable by hospital budgets. Their acquisition opportunity cost is a health policy matter requiring to display collaborative coping strategies with Ministries and other stakeholders including industry.

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## OP166 How Responsive Is Industry To Value Based Procurement?

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**INTRODUCTION:**

The European Union (EU) directive (2014/24) on public procurement strives to stimulate innovation and seeks for methodologies to implement a quality/cost based approach to search for the most economically advantageous tendering (MEAT). MedTech Europe launched the MEAT value-based-procurement (VBP) framework and tool which considers product's value from different perspectives/dimensions. Results from the first EU pilot, testing the feasibility to use the MEAT framework at a university hospital, are presented.