

are usually self-limiting and last on average for 4 to 7 days, with patients typically not accessing the public healthcare system (SUS). In severe cases, symptoms include neurological disorders and neonatal malformations. A future Zika vaccine can contribute to decreasing the number of cases and associated complications. However, this has to be balanced against continuing costs to control this and other vector borne diseases. Consequently, information about consumers' willingness to pay (WTP) for a hypothetical Zika vaccine can help with price setting discussions in Brazil starting with the private market before being considered within SUS.

**Methods.** A cross-sectional study was conducted among residents in one of the main provinces of Brazil (Minas Gerais) regarding their WTP for a hypothetical Zika vaccine with agreed characteristics. This included a mean effective protection of 80 percent, with the possibility of some local and systemic side-effects. The discussed price was USD 56.41 (BRL 180.00) per vaccination as this figure was utilized in a previous WTP study for a dengue vaccine.

**Results.** Five hundred and seventeen people were interviewed. However, thirty would not be vaccinated even if the vaccine was free. Most of the resultant interviewees (489) were female (58.2 percent), were employed (71.2 percent), had private health insurance (52.7 percent), had household incomes above twice the minimum wage (69.8 percent) and did not have Zika (96.9 percent). The median individual maximum WTP for this hypothetical Zika vaccine was USD 31.34 (BRL 100.00).

**Conclusions.** WTP research can contribute to decision-making about possible prices alongside other economic criteria once a Zika vaccine becomes available in Brazil alongside other programmes to control the virus.

## VP16 A NICE Way To Manage Managed Access: Case Study In Muscular Dystrophy

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**Introduction.** Managed access arrangements (MAAs) represent a way of enabling patient access to promising treatments while collecting real world data to inform future health technology evaluations (HTE) and commissioning decisions. In July 2016, the National Institute for Health and Care Excellence (NICE) recommended Ataluren for treating Duchenne Muscular Dystrophy within a MAA. NICE is uniquely placed to oversee the implementation and monitoring of this MAA in collaboration with multiple stakeholders to ensure the final outputs meet the needs of a future HTE.

**Methods.** NICE assembled an Ataluren Managed Access Oversight Committee (MAOC) consisting of representatives from the manufacturer, patient organisations, commissioning body and treatment centres. This group were to meet every six months under the chairmanship of NICE with the primary function of reviewing the progress of data collection and identifying operational challenges in implementing the terms of the arrangement.

**Results.** The Ataluren MAOC has convened four times since the MAA commenced and these discussions identified a number of important actions. Data completeness was a concern and

prompted stakeholders to collaborate on implementing measures to circumvent this, to ensure data quality for future HTE. Lack of awareness and understanding of the MAA in the patient community was highlighted and resulted in the production of lay information. A review of the statistical analysis plan resulted in the need for an agreement amendment. To ensure an audit trail and appropriate critique, NICE produced an amendment process to define and justify amendments made during the agreement term.

**Conclusions.** MAOC meetings play an important role in monitoring the progress of MAAs and have ensured that implementation issues are identified promptly and resolved with input from key stakeholders. This process allows NICE to coordinate the work of stakeholders to facilitate the success of the MAA, and will be adopted in future NICE MAAs in ultra-rare diseases.

## VP18 Potential Of Real World Evidence For 'IDEAL' Procedures Research

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**Introduction.** Randomized trials and similarly robust research methods generate evidence in carefully controlled settings, often with strict inclusion criteria. But patients in the 'real world' often have multiple comorbidities, and treatments are delivered within diverse environments. Trials are also difficult to fund, and rarely collect longitudinal data. Because of these, and other limitations, researchers are increasingly recognizing the inherent value of real world evidence (RWE). This is not only true for pharmaceutical products, and may have even more relevance in the evaluation of complex interventional procedures and non-medicines healthcare technologies. The Idea, Development, Exploration, Assessment, Learning (IDEAL) Framework guides the developmental 'pipeline' of surgical (and other) procedures, as well as medical device research (IDEAL-D). IDEAL informs the production of high-quality evidence of safety and effectiveness, but there is potential to further expand its applications.

**Methods.** Our aim is to investigate the feasibility of using of RWE alongside the IDEAL Framework in the assessment of procedures and devices. Methodological experts from the IDEAL Collaboration, HTA agencies and other healthcare research organisations are contributing their unique perspectives and experiences to explore these methods. As part of this work, Cedar Healthcare Technology Research Centre (Cedar) has attempted to retrospectively apply the IDEAL criteria to a series of RWE projects conducted on behalf of the National Institute for Health and Care Excellence (NICE) Interventional Procedures and Medical Technologies Evaluation Programmes.

**Results.** Cedar's experience indicates that there may be options for using retrospective routinely-collected linked data and other existing sources to address some of the requirements of IDEAL. Likewise, the IDEAL Framework is expected to be a helpful reference when designing new databases and clinical registries for prospective collection of relevant and informative evidence. Examples from several projects will be shared at the Health Technology Assessment International (HTAi) conference.