

'Serious Concerns' Raised by Watchdog Group on Cost-effectiveness of DMD Gene Therapies

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The Institute for Clinical and Economic Review (ICER) issued a draft report raising serious concerns about the cost-effectiveness of Sarepta's gene therapies Exondys 51 (eteplirsen), and the investigational golodirsen, as well as the corticosteroid Emflaza (deflazacort; by PTC Therapeutics), for treating Duchenne muscular dystrophy (DMD).

ICER's worries about these treatments come mostly from the limited evidence backing their efficacy or safety, and their current prices, which are considered too high for the potential health benefits they offer.

Public comments to this draft report are open until June 18. All comments will be taken into account to eventually revise the evaluation, and will be incorporated in the final report, scheduled for July 11, the institute announced in a press release. That "evidence report" will be subject to deliberation during a public meeting of the New England Comparative Effectiveness Public Advisory Council (New England CEPAC), one of ICER's three appraisal committees, on July 25.

ICER is a U.S. independent nonprofit organization, best known as the nation's independent watchdog on drug pricing. The agency provides comprehensive clinical and cost-effectiveness analyses of health treatments, tests, and procedures, and is funded by nonprofit foundations, drug makers, insurers, and government grants.

Before issuing the draft report, ICER incorporated the input it received from patients, clinicians, and other stakeholders.

ICER evaluated Emflaza's effectiveness based on the results of available clinical trials, involving a small number of patients, that compared Emflaza to prednisone, another commonly used corticosteroid.

"These data as well as additional observational [non-interventional] studies suggest that there may be some greater benefits on motor function with deflazacort, although not all data are consistent and the size of the benefits may be small," ICER reported.

Most data suggest less patient weight gain with Emflaza, but also reduced growth. Additionally, Emflaza has not been shown to improve pulmonary function outcomes compared with prednisone.

Another concern for ICER was the lack of sufficient evidence to say with confidence that Emflaza is safer than prednisone, especially in terms of behavioral and psychiatric side effects. This is an important aspect, as a main reason for interest in Emflaza was the potentially fewer risks of the treatment.

"Overall, given the evidence on motor function and weight, we have moderate certainty that deflazacort [Emflaza] has comparable or better net health benefits compared to prednisone," ICER said.

Concerning the two Sarepta gene therapies — Exondys 51 (eteplirsen) and golodirsen — ICER classified the available evidence on their clinical effectiveness as "insufficient."

Clinical data for both therapies is very limited, and studies of dystrophin levels show increases that are of "uncertain clinical or biologic importance," ICER said. Although Exondys 51 has not demonstrated any safety concerns, treated patients have thus far received follow-up only for short periods, which makes it difficult to evaluate long-term risks. For golodirsen, still an investigational drug, functional outcomes and safety data have not yet been reported.

"While there are important limitations to consider, ... the magnitudes of the treatment costs relative to the potential health effects projected for DMD suggest serious concerns regarding the cost-effectiveness of these treatments at current prices," ICER said.

Emflaza's incremental cost-effectiveness ratio was projected to stay above \$500,000 per quality-adjusted life-year.

For Exondys 51, ICER says it "would not be cost-effective," given its willingness-to-pay threshold of \$150,000 per quality-adjusted life-year, even when assuming the treatment will have extremely favorable efficacy.

Quality-adjusted life-year (QALY) is a measure of disease burden used in health economics, which corresponds to one year in perfect health.