Duchenne Population Potentially Amenable to Exon Skipping

Exon skipping is a potential treatment approach to correct and restore production of dystrophin. For specific genetic mutations it allows the body to make a shorter, usable dystrophin. Exon skipping is not a cure for Duchenne, but it may make the effects less severe. No single drug will help everyone with Duchenne. It is estimated that 13% of Duchenne patients have a mutation amenable to skipping exon 51. Exon skipping may eventually apply to 60-80 percent of Duchenne patients.

This document contains theoretical and documented mutations potentially amenable to exon skipping. Not all deletions have been studied and this list may not be complete. This is an educational resource to provide information about exon skipping only. Please contact your child’s physician or genetic counselor for more information.

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This document was prepared by CureDuchenne™.
