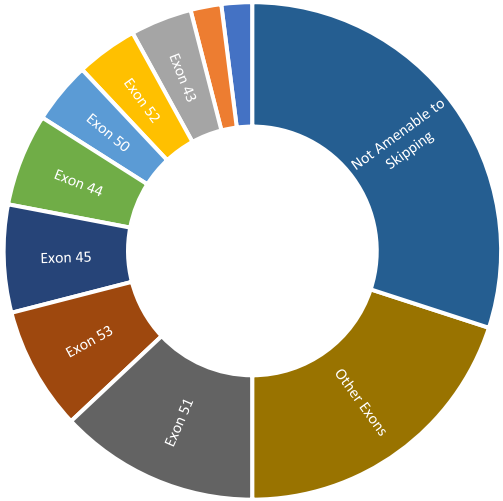
Duchenne Population Potentially Amenable to Exon Skipping

Exon skipping is a potential treatment approach to correct and restore production of dystophin. For specific genetic mutations it allows the body to make a shorter, usable dystophin. 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.



Exon 51 Skip-amenable

3-50	4-50	5-50	6-50	9-50					
10-50	11-50	13-50	14-50	15-50	16-50	17-50	19-50		
21-50	23-50	24-50	25-50	26-50	27-50	28-50	29-50		
30-50	31-50	32-50	33-50	34-50	35-50	36-50	37-50	38-50	39-50
40-50	41-50	42-50	43-50	45-50	47-50	48-50	49-50		
50	52	52-58	52-61	52-63	52-64	52-66	52-76	52-77	

Exon 53 Skip-amenable

3-52	4-52	5-52	6-52	9-52					
10-52	11-52	13-52	14-52	15-52	16-52	17-52	19-52		
21-52	23-52	24-52	25-52	26-52	27-52	28-52	29-52		
30-52	31-52	32-52	33-52	34-52	35-52	36-52	37-52	38-52	39-52
40-52	41-52	42-52	43-52	45-52	47-52	48-52	49-52		
50-52	52	54-58	54-61	54-63	54-64	54-66	54-76	54-77	

Exon 45 Skip-amenable

7-44				
12-44	18-44			
44	46	46-47	46-48	46-49
46-51	46-53	46-55	46-57	46-59
46-60	46-67	46-69	46-75	46-78

Exon 44 Skip-amenable

3-43	4-43	5-43	6-43	9-43					
10-43	11-43	13-43	14-43	15-43	16-43	17-43	19-43		
21-43	23-43	24-43	25-43	26-43	27-43	28-43	29-43		
30-43	31-43	32-43	33-43	34-43	35-43	36-43	37-43	38-43	39-43
40-43	41-43	42-43	43	45	45-54	45-56	45-62		
45-65	45-68	45-70	45-71	45-72	45-73	45-74			

Exon 50 Skip-amenable

2-49	8-49					
20-49	22-49					
51	51-53	51-55				
51-57	51-59	51-60	51-67	51-69	51-75	51-78

Exon 52 Skip-amenable

2-51	8-51				
20-51	22-51				
51	53	53-55	53-57	53-59	53-60
53-67	53-69	53-75	53-78		

Exon 43 Skip-amenable

7-42	12-42	18-42							
44	44-46	44-47	44-48	44-49	44-51	44-53	44-55	44-57	44-59

Exon 55 Skip-amenable

3-54	4-54	5-54	6-54	9-54					
10-54	11-54	13-54	14-54	15-54	16-54	17-54	19-54		
21-54	23-54	24-54	25-54	25-54	26-54	27-54	28-54	29-54	
30-54	31-54	32-54	33-54	34-54	35-54	36-54	37-54	38-54	39-54
40-54	41-54	42-54	43-54	45-54	47-54	48-54	49-54		
50-54	52-54	54	56	56-62					
56-65	56-68	56-70	56-71	56-72	56-73	56-74			

Exon 8 Skip-amenable

3-7	4-7	5-7	6-7	9-19	9-21
9-50	9-52	9-53			

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.
Duchenne population amenable to exon skipping was determined through the following source:
Fletcher, S., et. al. Dystrophin Isoform Induction In Vivo by Antisense-mediated Alternative Splicing. The American Society of Gene & Cell Therapy. 2010;18(6):1218-1223.
Annemieke Aartsma-Rus, et al. Theoretic applicability of antisense-mediated exon skipping for Duchenne muscular dystrophy. Hum Mutat. 2009 Mar;30 (3):293-9.

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