# **Supplementary information**

# A framework for individualized spliceswitching oligonucleotide therapy

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# Supplementary Fig. 1 (page 1/18)

Fig. 4



### Supplementary Fig. 1 (page 2/18)

#### Fig. 5a/Extended Data Figs. 6d,7b



Case (*ATM* -/c.7865C>T)



bp 1000 Normal splicing (826) ► Mis-splicing (762) ► 700

# Supplementary Fig. 1 (page 3/18)

#### Fig. 5a/Extended Data Fig. 7b







**bp** 1000

# Supplementary Fig. 1 (page 4/18)

#### Fig. 5a/Extended Data Fig. 7b



Normal splicing (826) Mis-splicing (762) 700

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#### Fig. 5a/Extended Data Fig. 7b



### Supplementary Fig. 1 (page 6/18)

#### Fig. 5a/Extended Data Fig. 7b



### Supplementary Fig. 1 (page 7/18)

#### Fig. 5b/Extended Data Fig. 8a



#### representative blots shown in Ext. Data Fig. 8a



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Fig. 5b/Extended Data Fig. 8a



### Supplementary Fig. 1 (page 9/18)

Extended Data Fig. 5



### Supplementary Fig. 1 (page 10/18)

#### Extended Data Fig. 8c - AT008





### Supplementary Fig. 1 (page 11/18)

#### Extended Data Fig. 8c - AT026

NTC Naïve NT-20 NT-22 0

1

2

5

10

20

50

100

200

500 1000



NTC Naïve NT-20 NT-22 0 1 2 5 10 20 50 100 200 500 1000



bp 1000 ▲ Normal splicing (826) 700 ▲ Mis-splicing (762)



# Supplementary Fig. 1 (page 12/18)

#### Extended Data Figs. 9c,10

#### Case (ATM -/c.5763-1050A>G)







Naïve1 Naïve2 Mock NT-22 NT-20 AT042 AT043 AT044 AT045 AT046 AT047 AT048 AT049 AT050 AT051 AT052 AT053 AT054

500 Mis-splicing (417) ► 400 Normal splicing (280) ► <sup>300</sup>

# Supplementary Fig. 1 (page 13/18)

#### Extended Data Fig. 10





# Supplementary Fig. 1 (page 14/18)

#### Extended Data Fig. 10



Case



| Case<br>( <i>ATM -/</i> c.5763-1050A>G) |       |       |       |       |       |       |  |
|---|-------|-------|-------|-------|-------|-------|--|
| AT063                                   | AT064 | AT065 | AT066 | AT067 | AT068 | AT043 |  |
|   |       |       |       |       |       |       |  |
|   |       |       |       |       |       |       |  |
|   |       |       |       |       |       |       |  |
|   |       |       |       |       |       |       |  |



# Supplementary Fig. 1 (page 15/18)

#### Extended Data Fig. 10



### Supplementary Fig. 1 (page 16/18)

#### Extended Data Fig. 10



Case

Case



### Supplementary Fig. 1 (page 17/18)

### Extended Data Fig. 10



Case (ATM -/c.5763-1050A>G)

Normal splicing (280)  $\blacktriangleright$  <sup>300</sup>



### Supplementary Fig. 1 (page 18/18)



representative blots shown in Ext. Data Fig. 11a



Supplementary Fig 1. Unprocessed gel and blot images