

4. 4. What do you see as the main obstacles to TREAT-NMD's continuation in the future?

	Response Count
	287
<i>answered question</i>	287
<i>skipped question</i>	143

5.

5.1

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Facilitating international collaborations between research groups to share data and experience and develop harmonized tools and protocols.	91.3% (337)	7.3% (27)	0.8% (3)	0.5% (2)	369
	<i>answered question</i>				369
	<i>skipped question</i>				61

6.

5.1.1 If you selected “activity should not continue”, please give your reason:

		Response Percent	Response Count
Work has already been achieved		75.0%	6
Too expensive		0.0%	0
Duplicates work done elsewhere		25.0%	2
Other (please specify)		0.0%	0
answered question			8
skipped question			422



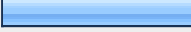
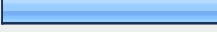
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5.2

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Evaluating existing animal models for NMDs, developing international consensus on their use and creating standard operating procedures (SOPs) to allow standardized assessment across labs www.treat-nmd.eu/animalmodels	42.8% (158)	48.8% (180)	3.8% (14)	4.6% (17)	369
answered question					369
skipped question					61

8.

5.2.1 If you selected “activity should not continue”, please give your reason:

		Response Percent	Response Count
Work has already been achieved		30.4%	7
Too expensive		4.3%	1
Duplicates work done elsewhere		30.4%	7
Other (please specify)		34.8%	8
<i>answered question</i>			23
<i>skipped question</i>			407



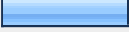
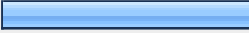
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5.3

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Facilitating global exchange of biomaterials for research via an online database of DNA, cell and tissue samples (EuroBioBank) www.treat-nmd.eu/eurobiobank	58.6% (215)	34.1% (125)	2.2% (8)	5.2% (19)	367
<i>answered question</i>					367
<i>skipped question</i>					63

10.

5.3.1 If you selected “activity should not continue”, please give your reason:

		Response Percent	Response Count
Work has already been achieved		33.3%	5
Too expensive		6.7%	1
Duplicates work done elsewhere		20.0%	3
Other (please specify)		40.0%	6
			answered question
			15
			skipped question
			415



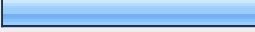
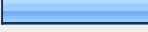
11.

5.4

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Acting as a platform for spin-off projects (bringing participants together in the set-up phase, assisting in proposal-writing, and acting as a communication and dissemination platform).	47.8% (175)	41.8% (153)	4.6% (17)	5.7% (21)	366
					answered question
					366
					skipped question
					64

12.

5.4.1 If you selected “activity should not continue”, please give your reason:

		Response Percent	Response Count
Work has already been achieved		29.4%	5
Too expensive		5.9%	1
Duplicates work done elsewhere		41.2%	7
Other (please specify)		23.5%	4
answered question			17
skipped question			413



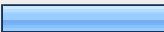
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5.5

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Providing expert evaluation and advice to researchers on the steps to be taken to take promising drugs and therapeutic targets through to clinical trial (TREAT-NMD Advisory Committee for Therapeutics, TACT) www.treat-nmd.eu/TACT	69.4% (249)	24.2% (87)	4.7% (17)	1.7% (6)	359
answered question					359
skipped question					71

14.

5.5.1 If you selected “activity should not continue”, please give your reason:

	Response Percent	Response Count
Work has already been achieved 	10.5%	2
Too expensive	0.0%	0
Duplicates work done elsewhere 	63.2%	12
Other (please specify) 	26.3%	5
<i>answered question</i>		19
<i>skipped question</i>		411



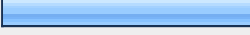
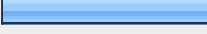
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5.6

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Providing expert support services for neuromuscular clinical trials, advising researchers, companies or CROs (Clinical Trial Coordination Centre, CTCC) www.treat-nmd.eu/ctcc	69.1% (246)	26.4% (94)	2.5% (9)	2.0% (7)	356
<i>answered question</i>					356
<i>skipped question</i>					74

16.

5.6.1 If you selected “activity should not continue”, please give your reason:

		Response Percent	Response Count
Work has already been achieved		13.3%	2
Too expensive		13.3%	2
Duplicates work done elsewhere		40.0%	6
Other (please specify)		33.3%	5
<i>answered question</i>			15
<i>skipped question</i>			415

17.

5.7

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Developing a global registry of care and trial sites specializing in NMDs including information about their facilities (Care and Trial Site Registry, CTSR) www.treat-nmd.eu/ctsr	67.6% (238)	28.1% (99)	3.1% (11)	1.1% (4)	352
<i>answered question</i>					352
<i>skipped question</i>					78

18.

5.7.1 If you selected “activity should not continue”, please give your reason:

		Response Percent	Response Count
Work has already been achieved		26.7%	4
Too expensive		6.7%	1
Duplicates work done elsewhere		46.7%	7
Other (please specify)		20.0%	3
<i>answered question</i>			15
<i>skipped question</i>			415


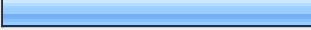

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5.8

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Developing patient registries to facilitate patient recruitment for trials and provide feedback to patients on developments relevant to their condition www.treat-nmd.eu/patientregistries	76.7% (277)	19.7% (71)	1.7% (6)	1.9% (7)	361
<i>answered question</i>					361
<i>skipped question</i>					69

20.

5.8.1 If you selected “activity should not continue”, please give your reason:

		Response Percent	Response Count
Work has already been achieved		16.7%	2
Too expensive		0.0%	0
Duplicates work done elsewhere		50.0%	6
Other (please specify)		33.3%	4
<i>answered question</i>			12
<i>skipped question</i>			418

21.

5.9

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Developing and validating outcome measures appropriate for clinical trials in different conditions www.treat-nmd.eu/outcome-measures	69.1% (248)	25.3% (91)	2.2% (8)	3.3% (12)	359
<i>answered question</i>					359
<i>skipped question</i>					71

22.

5.9.1 If you selected “activity should not continue”, please give your reason:

		Response Percent	Response Count
Work has already been achieved		23.1%	3
Too expensive		15.4%	2
Duplicates work done elsewhere		15.4%	2
Other (please specify)		46.2%	6
<i>answered question</i>			13
<i>skipped question</i>			417



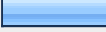
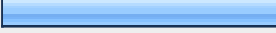
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5.10

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Developing an online Registry of Outcome Measures with active user input to facilitate access to existing measures www.treat-nmd.eu/rom	49.6% (177)	41.2% (147)	3.4% (12)	5.9% (21)	357
<i>answered question</i>					357
<i>skipped question</i>					73

24.

5.10.1 If you selected “activity should not continue”, please give your reason:

		Response Percent	Response Count
Work has already been achieved		27.8%	5
Too expensive		11.1%	2
Duplicates work done elsewhere		16.7%	3
Other (please specify)		44.4%	8
<i>answered question</i>			18
<i>skipped question</i>			412



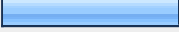
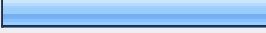
25.

5.11

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Engaging the regulatory authorities, such as EMA and FDA, in strategic dialogue on areas of key concern to NMDs.	59.9% (212)	31.4% (111)	2.8% (10)	5.9% (21)	354
<i>answered question</i>					354
<i>skipped question</i>					76

26.

5.11.1 If you selected “activity should not continue”, please give your reason:

		Response Percent	Response Count
Work has already been achieved		21.4%	3
Too expensive		7.1%	1
Duplicates work done elsewhere		28.6%	4
Other (please specify)		42.9%	6
<i>answered question</i>			14
<i>skipped question</i>			416




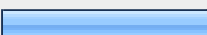
27.

5.12

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Maintaining a database of regulatory requirements for clinical trials in different countries www.treat-nmd.eu/regulatoryaffairs	43.1% (153)	43.1% (153)	7.3% (26)	6.5% (23)	355
<i>answered question</i>					355
<i>skipped question</i>					75

28.

5.12.1 If you selected “activity should not continue”, please give your reason:

		Response Percent	Response Count
Work has already been achieved		14.8%	4
Too expensive		14.8%	4
Duplicates work done elsewhere		37.0%	10
Other (please specify)		33.3%	9
<i>answered question</i>			27
<i>skipped question</i>			403

29.

5.13

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Supporting national networking of expert centres and patient organizations in countries worldwide with the aim of improving care standards and facilitating trial-readiness	69.2% (247)	26.1% (93)	2.8% (10)	2.0% (7)	357
<i>answered question</i>					357
<i>skipped question</i>					73

30.

5.13.1 If you selected “activity should not continue”, please give your reason:

		Response Percent	Response Count
Work has already been achieved		16.7%	2
Too expensive		16.7%	2
Duplicates work done elsewhere		41.7%	5
Other (please specify)		25.0%	3
<i>answered question</i>			12
<i>skipped question</i>			418





31.

5.14

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Undertaking and updating systematic reviews of published and unpublished clinical trials (Cochrane reviews).	43.0% (153)	43.5% (155)	7.3% (26)	6.2% (22)	356
<i>answered question</i>					356
<i>skipped question</i>					74

32.

5.14.1 If you selected “activity should not continue”, please give your reason:

		Response Percent	Response Count
Work has already been achieved		6.9%	2
Too expensive		13.8%	4
Duplicates work done elsewhere		62.1%	18
Other (please specify)		17.2%	5
<i>answered question</i>			29
<i>skipped question</i>			401

33.

5.15

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Developing and implementing international consensus guidelines on standards of care for NMDs www.treat-nmd.eu/care	69.6% (250)	24.0% (86)	4.7% (17)	1.7% (6)	359
<i>answered question</i>					359
<i>skipped question</i>					71

34.

5.15.1 If you selected “activity should not continue”, please give your reason:

		Response Percent	Response Count
Work has already been achieved		16.7%	4
Too expensive		12.5%	3
Duplicates work done elsewhere		29.2%	7
Other (please specify)		41.7%	10
			answered question
			24
			skipped question
			406

35.

5.16

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Developing and implementing international consensus guidelines on diagnostic standards and techniques for NMDs www.treat-nmd.eu/diagnosis	63.4% (227)	30.2% (108)	4.7% (17)	1.7% (6)	358
					answered question
					358
					skipped question
					72

36.

5.16.1 If you selected “activity should not continue”, please give your reason:

		Response Percent	Response Count
Work has already been achieved		26.1%	6
Too expensive		4.3%	1
Duplicates work done elsewhere		39.1%	9
Other (please specify)		30.4%	7
answered question			23
skipped question			407



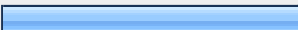
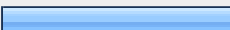
37.

5.17

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Support lobbying initiatives (providing expert opinions and information on best practice to influence national healthcare and research decisions).	43.1% (155)	43.1% (155)	6.9% (25)	6.9% (25)	360
answered question					360
skipped question					70

38.

5.17.1 If you selected “activity should not continue”, please give your reason:

		Response Percent	Response Count
Work has already been achieved		7.4%	2
Too expensive		7.4%	2
Duplicates work done elsewhere		48.1%	13
Other (please specify)		37.0%	10
<i>answered question</i>			27
<i>skipped question</i>			403

39.

5.18

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Hosting small focused workshops on a particular area where progress is required.	45.7% (163)	43.4% (155)	8.1% (29)	2.8% (10)	357
<i>answered question</i>					357
<i>skipped question</i>					73

40.

5.18.1 If you selected “activity should not continue”, please give your reason:

	Response Percent	Response Count
Work has already been achieved	2.9%	1
Too expensive	11.8%	4
Duplicates work done elsewhere	61.8%	21
Other (please specify)	23.5%	8
<i>answered question</i>		34
<i>skipped question</i>		396


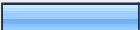
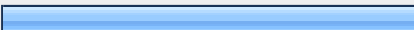

41.

5.19

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Hosting larger international conferences on broader issues in translational research in NMDs.	34.0% (121)	52.0% (185)	10.1% (36)	3.9% (14)	356
<i>answered question</i>					356
<i>skipped question</i>					74

42.

5.19.1 If you selected “activity should not continue”, please give your reason:

	Response Percent	Response Count
Work has already been achieved 	2.2%	1
Too expensive 	21.7%	10
Duplicates work done elsewhere 	67.4%	31
Other (please specify) 	8.7%	4
<i>answered question</i>		46
<i>skipped question</i>		384



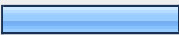
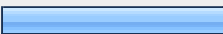
43.

5.20

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Providing expert training on NMDs to healthcare professionals in countries with poorer access to specialist services.	56.1% (198)	34.6% (122)	5.7% (20)	3.7% (13)	353
<i>answered question</i>					353
<i>skipped question</i>					77

44.

5.20.1 If you selected “activity should not continue”, please give your reason:

		Response Percent	Response Count
Work has already been achieved		7.1%	2
Too expensive		28.6%	8
Duplicates work done elsewhere		28.6%	8
Other (please specify)		35.7%	10
<i>answered question</i>			28
<i>skipped question</i>			402

45.

5.21

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Providing good clinical practice (GCP) training.	39.9% (141)	40.5% (143)	13.6% (48)	5.9% (21)	353
<i>answered question</i>					353
<i>skipped question</i>					77

46.

5.21.1 If you selected “activity should not continue”, please give your reason:

	Response Percent	Response Count
Work has already been achieved	5.6%	3
Too expensive	13.0%	7
Duplicates work done elsewhere	68.5%	37
Other (please specify)	13.0%	7
<i>answered question</i>		54
<i>skipped question</i>		376

47.

5.22

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Providing specialist training on trial design (special focus on NMD trials and the challenges of trials in small populations).	46.6% (166)	39.6% (141)	7.0% (25)	6.7% (24)	356
<i>answered question</i>					356
<i>skipped question</i>					74

48.

5.22.1 If you selected “activity should not continue”, please give your reason:

		Response Percent	Response Count
Work has already been achieved		10.7%	3
Too expensive		14.3%	4
Duplicates work done elsewhere		50.0%	14
Other (please specify)		25.0%	7
<i>answered question</i>			28
<i>skipped question</i>			402

49.

5.23

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Developing a standardized curriculum for specialist medical training in myology in Europe.	30.7% (109)	48.2% (171)	8.2% (29)	13.0% (46)	355
<i>answered question</i>					355
<i>skipped question</i>					75

50.

5.23.1 If you selected “activity should not continue”, please give your reason:

		Response Percent	Response Count
Work has already been achieved		11.1%	4
Too expensive		5.6%	2
Duplicates work done elsewhere		50.0%	18
Other (please specify)		33.3%	12
<i>answered question</i>			36
<i>skipped question</i>			394



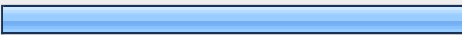

51.

5.24

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Maintaining a website providing independent expert information for patients and families, healthcare professionals, researchers, and industry www.treat-nmd.eu	68.2% (242)	26.5% (94)	3.9% (14)	1.4% (5)	355
<i>answered question</i>					355
<i>skipped question</i>					75

52.

5.24.1 If you selected “activity should not continue”, please give your reason:

		Response Percent	Response Count
Work has already been achieved		15.0%	3
Too expensive		5.0%	1
Duplicates work done elsewhere		75.0%	15
Other (please specify)		5.0%	1
answered question			20
skipped question			410

53.

5.25

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Providing online tools for collaboration between research groups (intranet, filesharing etc.)	50.8% (180)	36.2% (128)	7.1% (25)	5.9% (21)	354
answered question					354
skipped question					76

54.

5.25.1 If you selected “activity should not continue”, please give your reason:

	Response Percent	Response Count
Work has already been achieved	6.9%	2
Too expensive	3.4%	1
Duplicates work done elsewhere	55.2%	16
Other (please specify)	34.5%	10
<i>answered question</i>		29
<i>skipped question</i>		401

55.

5.26

	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Providing updates by means of regular newsletters www.treat-nmd.eu/newsletter	53.4% (189)	41.2% (146)	3.7% (13)	1.7% (6)	354
<i>answered question</i>					354
<i>skipped question</i>					76

56.

5.26.1 If you selected “activity should not continue”, please give your reason:

		Response Percent	Response Count
Work has already been achieved		15.4%	2
Too expensive		23.1%	3
Duplicates work done elsewhere		23.1%	3
Other (please specify)		38.5%	5
<i>answered question</i>			13
<i>skipped question</i>			417



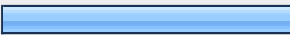
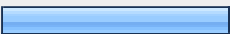
57.

5.27

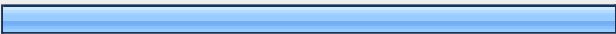
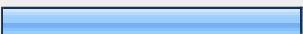
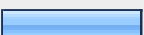


	Top priority	Secondary priority	Activity should not continue	No opinion	Response Count
Providing a forum for discussion and debate on social and ethical issues for both patients and professionals www.treat-nmd.eu/ethics	31.4% (111)	53.1% (188)	8.2% (29)	7.3% (26)	354
<i>answered question</i>					354
<i>skipped question</i>					76

58.

5.27.1 If you selected “activity should not continue”, please give your reason:

	Response Percent	Response Count
Work has already been achieved 	13.3%	4
Too expensive 	3.3%	1
Duplicates work done elsewhere 	46.7%	14
Other (please specify) 	36.7%	11
<i>answered question</i>		30
<i>skipped question</i>		400

59. 6.1 What other activities not mentioned above should TREAT-NMD focus on in future? Please detail the activity and explain why TREAT-NMD is the appropriate body.

	Response Percent	Response Count
One 	100.0%	107
Two 	48.6%	52
Three 	22.4%	24
Four 	8.4%	9
Five 	2.8%	3
<i>answered question</i>		107
<i>skipped question</i>		323

60. 7.1 What is your primary area of interest in neuromuscular disease? (Please indicate the disease or specific area of research or care you are most involved in.)

	Response Count
	298
<i>answered question</i>	298
<i>skipped question</i>	132

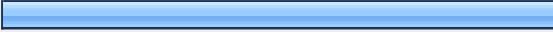

61. 7.2 Please summarize what needs to be achieved in your area of interest in (a) 1 year, (b) 3 years and (c) 5 years from now.



62. 7.3 How would you define the “roadmap” or sequence of concrete steps towards achieving the above goals?

	Response Count
	159
<i>answered question</i>	159
<i>skipped question</i>	271

63. 7.4 Are TREAT-NMD tools, services and infrastructure useful in the achievement of the above goals?

	Response Percent	Response Count
Yes 	90.2%	238
No 	9.8%	26
Please explain your response		154
<i>answered question</i>		264
<i>skipped question</i>		166

64. TREAT-NMD aims to promote international research collaborations and make it easier for different groups to share information and work together. What additional specific measures should TREAT-NMD put in place to facilitate effective international research collaborations in future?

	Response Count
	147
<i>answered question</i>	147
<i>skipped question</i>	283

65. 9. What specific measures should TREAT-NMD put in place to ensure its stakeholders have an adequate voice at a strategic level?

	Response Count
	138
<i>answered question</i>	138
<i>skipped question</i>	292

66. 10. What specific measures should TREAT-NMD put in place to ensure it retains its current independence?

	Response Count
	149
<i>answered question</i>	149
<i>skipped question</i>	281


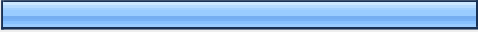
67. 11. What specific measures should TREAT-NMD put in place to ensure active ideas generation can come from anywhere within the neuromuscular field?

	Response Count
	153
<i>answered question</i>	153
<i>skipped question</i>	277

68. 12. What specific measures should TREAT-NMD put in place to ensure its initiatives are well integrated and harmonized with those of other stakeholders active in the field?

	Response Count
	124
<i>answered question</i>	124
<i>skipped question</i>	306

69. 13. One possible model for the future structure envisages the creation of a board of directors to provide oversight and an executive committee made up of representatives of the institutions carrying out the work. In such a model, should strategic decisions be taken by:

	Response Percent	Response Count
13.1 The board of directors 	10.7%	26
13.2 The executive committee 	11.9%	29
13.3 Both bodies, with respective responsibilities defined contractually 	77.5%	189
Please provide any further comments on responsibility for strategic decisions here.		40
<i>answered question</i>		244
<i>skipped question</i>		186

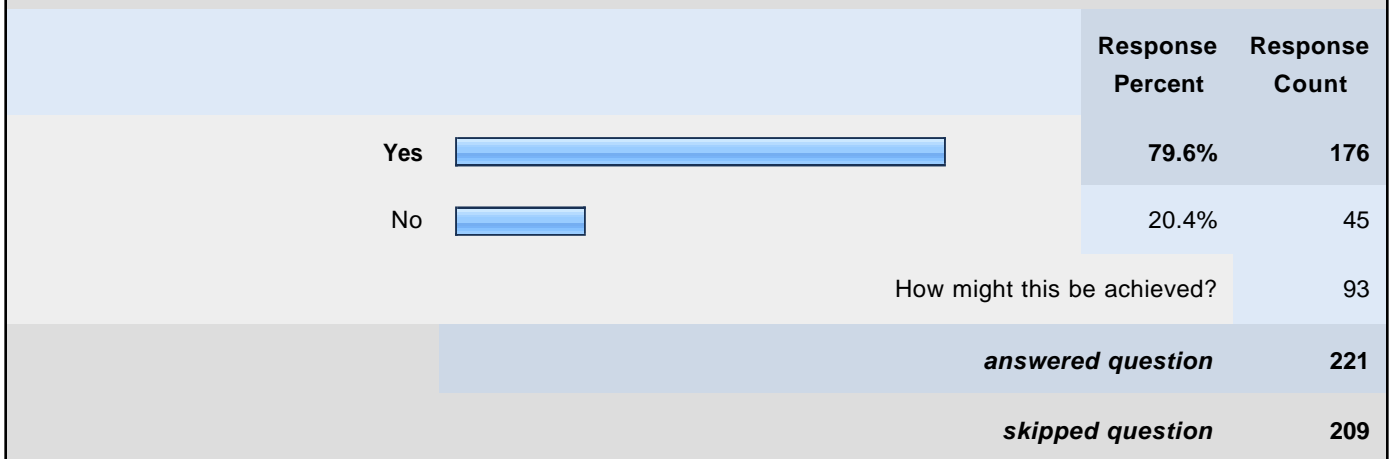
70. 14. If you have any other comments about the future governance and oversight structure, please provide them here.

	Response Count
	39
<i>answered question</i>	39
<i>skipped question</i>	391

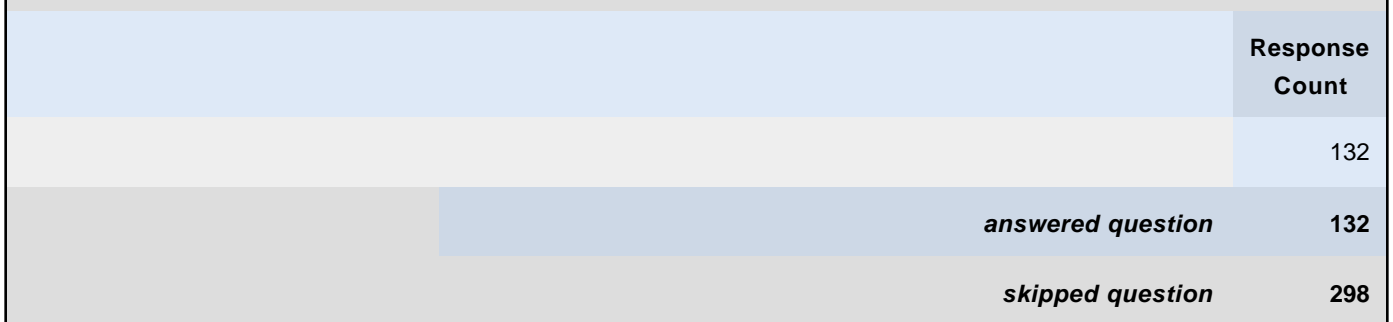
71. 16.1 What rights/benefits should such funders expect as a return on their investment?

	Response Count
	177
<i>answered question</i>	177
<i>skipped question</i>	253

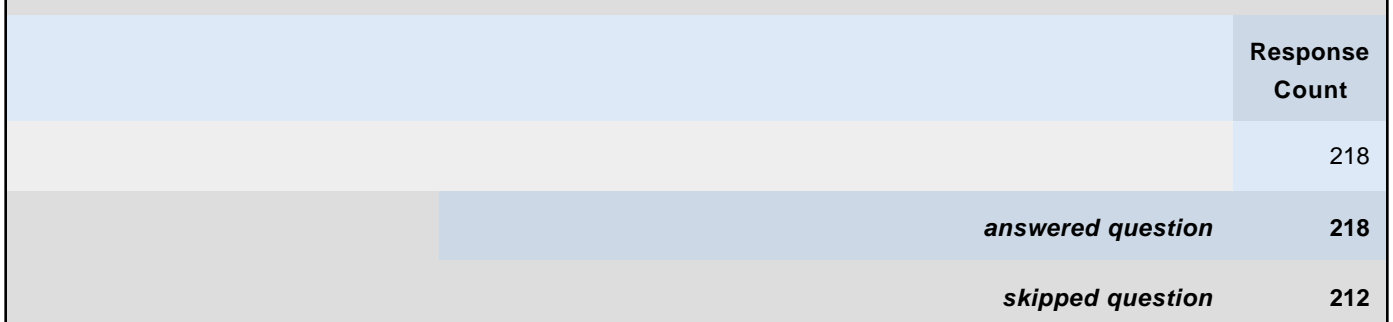
72. 16.2 Is there a need to enable organizations to provide project-specific funding targeted at individual TREAT-NMD activities?



73. 16.3 How might funding to sustain the overall infrastructure and services, including management, coordination and administrative activities, be obtained?



74. 17.1 Should TREAT-NMD ask industry to pay for services?



75. 17.2 Should industry sponsor meetings associated with TREAT-NMD?

	Response Count
	217
<i>answered question</i>	217
<i>skipped question</i>	213

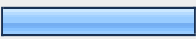
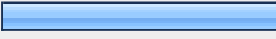
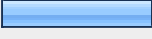
76. 17.3 Would it be appropriate for industry to contribute to the future funding of TREAT-NMD in other ways? If so, which? If not, why not?

	Response Count
	163
<i>answered question</i>	163
<i>skipped question</i>	267

77. 17.4 How else could TREAT-NMD develop its services for industry?

	Response Count
	93
<i>answered question</i>	93
<i>skipped question</i>	337

78. 18.1 Should TREAT-NMD consider initiating membership fees to cover some of its operational costs (as many professional societies and charitable bodies do)?

	Response Percent	Response Count
Immediately after the end of the EU funding 	31.1%	80
Not immediately but maybe in a few more years 	44.7%	115
Never 	24.1%	62
Please explain your response		147
answered question		257
skipped question		173

79. 19.1 Please provide any other comments about the future funding of TREAT-NMD here.

	Response Count
	46
answered question	46
skipped question	384







80. 20. Is there anything else you'd like to tell us about your views on the future of TREAT-NMD?

	Response Count
	47
answered question	47
skipped question	383









81. 21.1 Name	
	Response Count
	235
<i>answered question</i>	235
<i>skipped question</i>	195

82. 21.2 Email Address	
	Response Count
	236
<i>answered question</i>	236
<i>skipped question</i>	194

83. 21.3 Country in which you are living at present.

	Response Percent	Response Count
ac – Ascension Island	0.0%	0
ad – Andorra	0.0%	0
ae – United Arab Emirates	0.0%	0
af – Afghanistan	0.0%	0
ag – Antigua and Barbuda	0.0%	0
ai – Anguilla	0.0%	0
al – Albania	0.0%	0
am – Armenia	0.0%	0
an – Netherlands Antilles	0.0%	0
ao – Angola	0.0%	0
aq – Antarctica	0.0%	0
ar – Argentina 	0.7%	2
as – American Samoa	0.0%	0
at – Austria 	1.9%	5
au – Australia 	5.2%	14
aw – Aruba	0.0%	0
ax – Aland Islands 	0.4%	1
az – Azerbaijan	0.0%	0
ba – Bosnia and Herzegovina	0.0%	0
bb – Barbados	0.0%	0
bd – Bangladesh	0.0%	0
be – Belgium 	1.9%	5
bf – Burkina Faso	0.0%	0
bg – Bulgaria 	0.4%	1
bh – Bahrain	0.0%	0
bi – Burundi	0.0%	0
bj – Benin	0.0%	0








bm – Bermuda	0.0%	0
bn – Brunei Darussalam	0.0%	0
bo – Bolivia	0.0%	0
br – Brazil 	2.6%	7
bs – Bahamas	0.0%	0
bt – Bhutan	0.0%	0
bv – Bouvet Island	0.0%	0
bw – Botswana	0.0%	0
by – Belarus	0.0%	0
bz – Belize	0.0%	0
ca – Canada 	1.5%	4
cc – Cocos (Keeling) Islands	0.0%	0
cd – Congo, The Democratic Republic of the	0.0%	0
cf – Central African Republic	0.0%	0
cg – Congo, Republic of	0.0%	0
ch – Switzerland 	1.9%	5
ci – Cote d'Ivoire	0.0%	0
ck – Cook Islands	0.0%	0
cl – Chile	0.0%	0
cm – Cameroon	0.0%	0
cn – China 	0.4%	1
co – Colombia	0.0%	0
cr – Costa Rica	0.0%	0
cu – Cuba	0.0%	0
cv – Cape Verde	0.0%	0
cx – Christmas Island	0.0%	0
cy – Cyprus	0.0%	0
cz – Czech Republic 	1.9%	5

de – Germany		10.7%	29
dj – Djibouti		0.0%	0
dk – Denmark		0.7%	2
dm – Dominica		0.0%	0
do – Dominican Republic		0.0%	0
dz – Algeria		0.0%	0
ec – Ecuador		0.0%	0
ee – Estonia		0.0%	0
eg – Egypt		0.0%	0
eh – Western Sahara		0.0%	0
er – Eritrea		0.0%	0
es – Spain		3.3%	9
et – Ethiopia		0.0%	0
eu – European Union		0.7%	2
fi – Finland		1.1%	3
fj – Fiji		0.0%	0
fk – Falkland Islands (Malvinas)		0.0%	0
fm – Micronesia, Federated States of		0.0%	0
fo – Faroe Islands		0.0%	0
fr – France		4.1%	11
ga – Gabon		0.0%	0
gb – United Kingdom		0.4%	1
gd – Grenada		0.0%	0
ge – Georgia		0.4%	1
gf – French Guiana		0.0%	0
gg – Guernsey		0.0%	0
gh – Ghana		0.0%	0
gi – Gibraltar		0.0%	0
gl – Greenland		0.0%	0

gm – Gambia	0.0%	0
gn – Guinea	0.0%	0
gp – Guadeloupe	0.0%	0
gq – Equatorial Guinea	0.0%	0
gr – Greece	0.4%	1
gs – South Georgia and the South Sandwich Islands	0.0%	0
gt – Guatemala	0.0%	0
gu – Guam	0.0%	0
gw – Guinea-Bissau	0.0%	0
gy – Guyana	0.0%	0
hk – Hong Kong	0.0%	0
hm – Heard and McDonald Islands	0.0%	0
hn – Honduras	0.0%	0
hr – Croatia/Hrvatska	0.7%	2
ht – Haiti	0.0%	0
hu – Hungary	0.7%	2
id – Indonesia	0.0%	0
ie – Ireland	0.7%	2
il – Israel	1.5%	4
im – Isle of Man	0.0%	0
in – India	1.9%	5
io – British Indian Ocean Territory	0.0%	0
iq – Iraq	0.0%	0
ir – Iran, Islamic Republic of	0.4%	1
is – Iceland	0.0%	0
it – Italy	8.9%	24
je – Jersey	0.0%	0
jm – Jamaica	0.4%	1
jo – Jordan	0.0%	0

jp – Japan	0.4%	1
ke – Kenya	0.0%	0
kg – Kyrgyzstan	0.0%	0
kh – Cambodia	0.0%	0
ki – Kiribati	0.0%	0
km – Comoros	0.0%	0
kn – Saint Kitts and Nevis	0.0%	0
kp – Korea, Democratic People's Republic	0.0%	0
kr – Korea, Republic of	0.0%	0
kw – Kuwait	0.0%	0
ky – Cayman Islands	0.0%	0
kz – Kazakhstan	0.0%	0
la – Lao People's Democratic Republic	0.0%	0
lb – Lebanon	0.0%	0
lc – Saint Lucia	0.0%	0
li – Liechtenstein	0.0%	0
lk – Sri Lanka	0.0%	0
lr – Liberia	0.0%	0
ls – Lesotho	0.0%	0
lt – Lithuania	0.7%	2
lu – Luxembourg	0.0%	0
lv – Latvia	0.4%	1
ly – Libyan Arab Jamahiriya	0.0%	0
ma – Morocco	0.0%	0
mc – Monaco	0.0%	0
md – Moldova, Republic of	0.0%	0
me – Montenegro	0.0%	0
mg – Madagascar	0.0%	0
mh – Marshall Islands	0.0%	0

mk – Macedonia, The Former Yugoslav Republic of	0.4%	1
ml – Mali	0.0%	0
mm – Myanmar	0.0%	0
mn – Mongolia	0.0%	0
mo – Macao	0.0%	0
mp – Northern Mariana Islands	0.0%	0
mq – Martinique	0.0%	0
mr – Mauritania	0.0%	0
ms – Montserrat	0.0%	0
mt – Malta	0.0%	0
mu – Mauritius	0.0%	0
mv – Maldives	0.0%	0
mw – Malawi	0.0%	0
mx – Mexico	0.4%	1
my – Malaysia	0.0%	0
mz – Mozambique	0.0%	0
na – Namibia	0.0%	0
nc – New Caledonia	0.0%	0
ne – Niger	0.0%	0
nf – Norfolk Island	0.0%	0
ng – Nigeria	0.0%	0
ni – Nicaragua	0.0%	0
nl – Netherlands	4.8%	13
no – Norway	0.7%	2
np – Nepal	0.0%	0
nr – Nauru	0.0%	0
nu – Niue	0.0%	0
nz – New Zealand	0.7%	2
om – Oman	0.0%	0

pa – Panama	0.0%	0
pe – Peru	0.0%	0
pf – French Polynesia	0.0%	0
pg – Papua New Guinea	0.0%	0
ph – Philippines	0.0%	0
pk – Pakistan	0.0%	0
pl – Poland 	1.1%	3
pm – Saint Pierre and Miquelon	0.0%	0
pn – Pitcairn Island	0.0%	0
pr – Puerto Rico	0.0%	0
ps – Palestinian Territory, Occupied	0.0%	0
pt – Portugal 	1.1%	3
pw – Palau	0.0%	0
py – Paraguay	0.0%	0
qa – Qatar	0.0%	0
re – Reunion Island	0.0%	0
ro – Romania 	1.1%	3
rs – Serbia 	0.4%	1
ru – Russian Federation 	1.9%	5
rw – Rwanda	0.0%	0
sa – Saudi Arabia	0.0%	0
sb – Solomon Islands	0.0%	0
sc – Seychelles	0.0%	0
sd – Sudan	0.0%	0
se – Sweden 	1.9%	5
sg – Singapore	0.0%	0
sh – Saint Helena	0.0%	0
si – Slovenia 	0.4%	1
sj – Svalbard and Jan Mayen Islands	0.0%	0

sk – Slovak Republic	0.0%	0
sl – Sierra Leone	0.0%	0
sm – San Marino	0.0%	0
sn – Senegal	0.0%	0
so – Somalia	0.0%	0
sr – Suriname	0.0%	0
st – Sao Tome and Principe	0.0%	0
su – Soviet Union	0.0%	0
sv – El Salvador	0.0%	0
sy – Syrian Arab Republic	0.0%	0
sz – Swaziland	0.0%	0
tc – Turks and Caicos Islands	0.0%	0
td – Chad	0.0%	0
tf – French Southern Territories	0.0%	0
tg – Togo	0.0%	0
th – Thailand	0.4%	1
tj – Tajikistan	0.0%	0
tk – Tokelau	0.0%	0
tl – Timor-Leste	0.0%	0
tm – Turkmenistan	0.0%	0
tn – Tunisia	0.0%	0
to – Tonga	0.0%	0
tp – East Timor	0.0%	0
tr – Turkey	3.0%	8
tt – Trinidad and Tobago	0.0%	0
tv – Tuvalu	0.0%	0
tw – Taiwan	0.4%	1
tz – Tanzania	0.0%	0
ua – Ukraine	0.4%	1

ug – Uganda	0.0%	0
uk – United Kingdom	8.9%	24
um – United States Minor Outlying Islands	0.0%	0
us – United States	14.8%	40
uy – Uruguay	0.0%	0
uz – Uzbekistan	0.0%	0
va – Holy See (Vatican City State)	0.0%	0
vc – Saint Vincent and the Grenadines	0.0%	0
ve – Venezuela	0.0%	0
vg – Virgin Islands, British	0.0%	0
vi – Virgin Islands, U.S.	0.0%	0
vn – Vietnam	0.0%	0
vu – Vanuatu	0.0%	0
wf – Wallis and Futuna Islands	0.0%	0
ws – Samoa	0.0%	0
ye – Yemen	0.0%	0
yt – Mayotte	0.0%	0
yu – Yugoslavia	0.4%	1
za – South Africa	0.0%	0
zm – Zambia	0.0%	0
zw – Zimbabwe	0.0%	0
answered question		270
skipped question		160

**84. 22.1 Which of the following best describes your current role?
(Please tick all that apply)**

		Response Percent	Response Count
22.1.1 Scientist	<input type="checkbox"/>	39.6%	113
22.1.2 Clinician	<input checked="" type="checkbox"/>	44.2%	126
22.1.3 Other healthcare professional	<input type="checkbox"/>	9.5%	27
22.1.4 Affected person/patient	<input type="checkbox"/>	4.9%	14
22.1.5 Parent or other relative	<input type="checkbox"/>	15.8%	45
22.1.6 Patient organization representative	<input type="checkbox"/>	16.1%	46
22.1.7 Industry representative	<input type="checkbox"/>	2.1%	6
22.1.8 Government/health authority/regulatory representative	<input type="checkbox"/>	1.8%	5
	22.1.9 Other (please specify)		25
answered question			285
skipped question			145

85. 22.2 Which of the following best describes the type of organization you represent? (Please tick one box only)

	Response Percent	Response Count
22.2.1 Academic <input checked="" type="checkbox"/>	40.7%	118
22.2.2 Clinical <input checked="" type="checkbox"/>	24.8%	72
22.2.3 Biotech company <input type="checkbox"/>	2.8%	8
22.2.4 Large pharma <input type="checkbox"/>	0.7%	2
22.2.5 Learned society <input type="checkbox"/>	0.3%	1
22.2.6 Public sector funder <input type="checkbox"/>	1.7%	5
22.2.7 Charitable funder <input type="checkbox"/>	3.8%	11
22.2.8 Patient group <input checked="" type="checkbox"/>	19.3%	56
22.2.9 Government <input type="checkbox"/>	0.7%	2
22.2.10 Other (please specify) <input type="checkbox"/>	5.2%	15
answered question		290
skipped question		140

86. 22.3 Which of the following best describes your current role? (Please tick all that apply)

	Response Percent	Response Count
22.3.1 Basic research <input checked="" type="checkbox"/>	33.0%	93
22.3.2 Clinical trials <input checked="" type="checkbox"/>	35.5%	100
22.3.3 Patient care <input checked="" type="checkbox"/>	54.6%	154
22.3.4 Ethics and social issues <input type="checkbox"/>	9.6%	27
22.3.5 Translational medicine <input checked="" type="checkbox"/>	24.5%	69
22.3.6 Regulatory issues <input type="checkbox"/>	6.4%	18
22.3.7 Other (please specify) <input type="checkbox"/>	14.9%	42
answered question		282
skipped question		148