

SUPPLEMENTARY MATERIAL

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1 Statistical Analysis of the survey

1.1 Please indicate your knowledge level on cystic fibrosis

Sample size		401	
Margin of error		4.8%	
Mean		2.48	
Standard deviation		0.852	
Percentiles	¼	2 (good knowledge)	
	½	2 (good knowledge)	
	¾	3 (high knowledge)	
Binomial test		Test ratio	P-value
		0.50	0.028
Kolmogorov-Smirnov		Test ratio	P-value
		0.370	0.000
Shapiro-Wilk		Test ratio	P-value
		0.632	0.000

1.2 Which of the following therapeutic options is most likely to be successful in treating cystic fibrosis in the next 15 years?

Therapy	Number of responses
CFTR modulator Therapies	288
Genetic Therapies	18
Other Therapies	95

Sample size		401	
Margin of error		4.8%	
Mean		3.38	
Standard deviation		0.822	
Percentiles	¼	2 (Genetic therapies)	
	½	3 (CFTR modulator therapies)	
	¾	3 (CFTR modulator therapies)	
Mann-Whitney U		Statistic	P-value
		-1.996	0.046
Wilcoxon (H0: median = 3)		Statistic	P-value
		-0.780	0.435
Kolmogorov-Smirnov		Statistic	P-value
		0.447	0.000
Shapiro-Wilk		Statistic	P-value
		0.577	0.000

		Other Therapies	Genetic Therapies	CFTR Modulator Therapies	Total
Good knowledge	Number of observations	14	50	151	223
	Frequency	6.28%	22.42%	67.71%	100.0%

High Knowledge	Number of observations	9	29	137	178
	Frequency	5.06%	16.29%	76.97%	100.0%

1.3 Respondents who selected CFTR modulator therapies as the most likely to be successful in treating CF in the next 15 years

1.3.1 Please rank the following approaches from most likely to least likely to be successful in treating cystic fibrosis in the next 15 years

	Most Likely (1)	2	3	Least likely (4)
Potentiators	25.50%	51.40%	14.60%	8.50%
Correctors	57.00%	23.30%	9.20%	10.40%
Amplifiers	8.30%	12.50%	42.90%	36.30%
Stabilizers	9.10%	12.40%	34.00%	44.40%

		Potentiators	Correctors	Amplifiers	Stabilizers
Mann-Whitney U	Statistic	-2.080	-2.114	-0.223	-3.840
	P-value	0.037	0.035	0.823	0.000

Potentiators						
		1	2	3	4	Total
Good knowledge	Number of observations	29	53	25	13	120
	Frequency	24.2%	44.2%	20.8%	10.8%	100.0%
High Knowledge	Number of observations	34	74	11	8	127
	Frequency	26.8%	58.3%	8.7%	6.3%	100.0%

Correctors						
		1	2	3	4	Total
Good knowledge	Number of observations	62	28	15	16	121
	Frequency	51.2%	23.1%	12.4%	13.2%	100.0%
High Knowledge	Number of observations	80	30	8	10	128
	Frequency	62.5%	23.4%	6.3%	7.8%	100.0%

Amplifiers						
		1	2	3	4	Total
Good knowledge	Number of observations	10	18	42	46	116
	Frequency	8.6%	15.5%	36.2%	39.7%	100.0%
High Knowledge	Number of observations	10	12	61	41	124
	Frequency	8.1%	9.7%	49.2%	33.1%	100.0%

Stabilizers						
		1	2	3	4	Total
Good knowledge	Number of observations	19	19	37	41	116
	Frequency	16.4%	16.4%	31.9%	35.3%	100.0%
High Knowledge	Number of observations	3	11	45	66	125
	Frequency	2.4%	8.8%	36.0%	52.8%	100.0%

1.3.2 Fixing or replacing the CFTR gene will lead to a cure for cystic fibrosis

Sample size		252	
Margin of error		6.1%	
Mean		2.86	
Standard deviation		1.00	
Percentiles	¼	2 (Unlikely)	
	½	3 (likely after 15 years)	
	¾	4 (likely before 15 years)	
Mann-Whitney U		Statistic	P-value
		-0.429	0.668
Wilcoxon (H0: median = 3)		Statistic	P-value
		1.252	0.210
Kolmogorov-Smirnov		Statistic	P-value
		0.303	0.000
Shapiro-Wilk		Statistic	P-value
		0.812	0.000

		Unknown	Unlikely	Likely after 15 years	Likely before 15 years	Total
Good knowledge	Number of observations	25	9	54	35	123
	Frequency	20.3%	7.3%	43.9%	28.5%	100.0%
High Knowledge	Number of observations	16	14	65	34	129
	Frequency	12.4%	10.9%	50.4%	26.4%	100.0%

1.3.3 Please rank the following approaches from most likely to least likely to be successful in fixing or replacing the malfunctioning CFTR gene in the next 15 years

	Most likely (1)	2	3	Least likely (4)
CRISPR-Cas9	72.70%	13.00%	3.90%	10.40%
TALENs	14.80%	43.60%	26.80%	14.80%
Meganucleases	8.10%	11.50%	35.80%	44.60%
Zinc Finger Nucleases (ZFNs)	5.40%	31.10%	34.50%	29.10%

		CRISPR-Cas9	TALENs	Meganucleases	Zinc Finger Nucleases (ZFNs)
Mann-Whitney U	Statistic	-1.775	-1.431	-.122	-.342
	P-value	0.076	0.152	0.903	0.732

CRISPR-Cas9						
		1	2	3	4	Total
Good knowledge	Number of observations	47	11	2	11	71
	Frequency	66.2%	15.5%	2.8%	15.5%	100.0%
High Knowledge	Number of observations	65	9	4	5	83
	Frequency	78.3%	10.8%	4.8%	6.0%	100.0%

TALENs						
		1	2	3	4	Total
Good knowledge	Number of observations	12	31	16	8	67
	Frequency	17.9%	46.3%	23.9%	11.9%	100.0%
High Knowledge	Number of observations	10	34	24	14	82
	Frequency	12.2%	41.5%	29.3%	17.1%	100.0%

Meganucleases						
		1	2	3	4	Total
Good knowledge	Number of observations	7	6	25	30	68
	Frequency	10.3%	8.8%	36.8%	44.1%	100.0%
High Knowledge	Number of observations	5	11	28	36	80
	Frequency	6.3%	13.8%	35.0%	45.0%	100.0%

Zinc Finger Nucleases (ZFNs)						
		1	2	3	4	Total
Good knowledge	Number of observations	5	19	25	18	67
	Frequency	7.5%	28.4%	37.3%	26.9%	100.0%
High Knowledge	Number of observations	3	27	26	25	81
	Frequency	3.7%	33.3%	32.1%	30.9%	100.0%

1.4 Respondents who selected genetic therapies as the most likely to be successful in treating CF in the next 15 years

1.4.1 Please rank the following therapies from most likely to least likely to be successful in treating CF in the next 15 years

	Most likely (1)	2	3	4	Least likely (5)
Adeno-associated viruses (AAVs)-based therapies	17.70%	16.10%	25.80%	21.00%	19.40%
Lentiviruses-based therapies	9.40%	17.20%	15.60%	35.90%	21.90%
Liposome-based therapies	19.70%	21.20%	19.70%	9.10%	30.30%
Messenger RNA (mRNA)-based therapies	43.90%	24.20%	18.20%	9.10%	4.50%
Transfer RNA (tRNA)-based therapies	12.30%	23.10%	20.00%	23.10%	21.50%

		Adeno-associated viruses (AAVs)-based therapies	Lentiviruses-based therapies	Liposome-based therapies	Messenger RNA (mRNA)-based therapies	Transfer RNA (tRNA)-based therapies
Mann-Whitney U	Statistic	-2.120	-0.232	-1.067	-0.055	-1.144
	P-value	0.034	0.816	0.286	0.956	0.253

Adeno-associated viruses (AAVs)-based therapies							
		Most likely 1	2	3	4	Least likely 5	Total
Good knowledge	Number of observations	8	5	14	7	3	37
	Frequency	21.6%	13.5%	37.8%	18.9%	8.1%	100.0%
High Knowledge	Number of observations	3	5	2	6	9	25
	Frequency	12.0%	20.0%	8.0%	24.0%	36.0%	100.0%

Lentiviruses-based therapies							
		Most likely 1	2	3	4	Least likely 5	Total
Good knowledge	Number of observations	3	8	5	13	8	37
	Frequency	8.1%	21.6%	13.5%	35.1%	21.6%	100.0%
High Knowledge	Number of observations	3	3	5	10	6	27

	Frequency	11.1%	11.1%	18.5%	37.0%	22.2%	100.0%
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Liposome-based therapies							
		Most likely 1	2	3	4	Least likely 5	Total
Good knowledge	Number of observations	5	9	7	5	12	38
	Frequency	13.2%	23.7%	18.4%	13.2%	31.6%	100.0%
High Knowledge	Number of observations	8	5	6	1	8	28
	Frequency	28.6%	17.9%	21.4%	3.6%	28.6%	100.0%

Messenger RNA (mRNA)-based therapies							
		Most likely 1	2	3	4	Least likely 5	Total
Good knowledge	Number of observations	18	8	7	4	2	39
	Frequency	46.2%	20.5%	17.9%	10.3%	5.1%	100.0%
High Knowledge	Number of observations	11	8	5	2	1	27
	Frequency	40.7%	29.6%	18.5%	7.4%	3.7%	100.0%

Transfer RNA (tRNA)-based therapies							
		Most likely 1	2	3	4	Least likely 5	Total
Good knowledge	Number of observations	5	9	5	8	12	39
	Frequency	12.8%	23.1%	12.8%	20.5%	30.8%	100.0%
High Knowledge	Number of observations	3	6	8	7	2	26
	Frequency	11.5%	23.1%	30.8%	26.9%	7.7%	100.0%

1.4.2 Fixing or replacing the CFTR gene will lead to a cure for cystic fibrosis

Sample size		67	
Margin of error		11.9%	
Mean		3.46	
Standard deviation		0.893	
Percentiles	¼	3 (likely after 15 years)	
	½	4 (likely before 15 years)	
	¾	4 (likely before 15 years)	
Mann-Whitney U		Statistic	P-value
		-1.748	0.080
Wilcoxon (H0: median = 4)		Statistic	P-value
		1.310	0.190
Kolmogorov-Smirnov		Statistic	P-value
		0.383	0.000
Shapiro-Wilk		Statistic	P-value
		0.639	0.000

		Unknown	Unlikely	Likely after 15 years	Likely before 15 years	Total
Good knowledge	Number of observations	5	3	8	23	39
	Frequency	12.8%	7.7%	20.5%	59.0%	100.0%
High Knowledge	Number of observations	0	0	7	21	28
	Frequency	0.0%	0.0%	25.0%	75.0%	100.0%

1.4.3 Please rank the following approaches from most likely to least likely to be successful in fixing or replacing the malfunctioning CFTR gene in the next 15 years

	Most likely (1)	2	3	Least likely (4)
CRISPR-Cas9	78.70%	4.90%	6.60%	9.80%
TALENs	12.70%	52.70%	25.50%	9.10%
Meganucleases	5.40%	21.40%	28.60%	44.60%
Zinc Finger Nucleases (ZFNs)	5.40 %	21.40 %	37.50%	35.70%

		CRISPR- Cas9	TALENs	Meganucleases	Zinc Finger Nucleases (ZFNs)
Mann- Whitney U	Statistic	-0.516	-0.305	-0.289	-1.143
	P-value	0.606	0.760	0.772	0.253

CRISPR-Cas9						
		1	2	3	4	Total
Good knowledge	Number of observations	25	2	3	3	33
	Frequency	75.8%	6.1%	9.1%	9.1%	100.0%
High Knowledge	Number of observations	23	1	1	3	28
	Frequency	82.1%	3.6%	3.6%	10.7%	100.0%

TALENs						
		1	2	3	4	Total
Good knowledge	Number of observations	5	13	9	3	30
	Frequency	16.7%	43.3%	30.0%	10.0%	100.0%
High Knowledge	Number of observations	2	16	5	2	25
	Frequency	8.0%	64.0%	20.0%	8.0%	100.0%

Meganucleases						
		1	2	3	4	Total

Good knowledge	Number of observations	2	7	6	15	30
	Frequency	6.7%	23.3%	20.0%	50.0%	100.0%
High Knowledge	Number of observations	1	5	10	10	26
	Frequency	3.8%	19.2%	38.5%	38.5%	100.0%

Zinc Finger Nucleases (ZFNs)						
		1	2	3	4	Total
Good knowledge	Number of observations	1	9	12	9	31
	Frequency	3.2%	29.0%	38.7%	29.0%	100.0%
High Knowledge	Number of observations	2	3	9	11	25
	Frequency	8.0%	12.0%	36.0%	44.0%	100.0%

1.5 Respondents who selected 'other' option as the most likely to be successful in treating CF in the next 15 years

1.5.1 Fixing or replacing the CFTR gene will lead to a cure for cystic fibrosis

Sample size		24	
Margin of error		20.0%	
Mean		2.67	
Standard deviation		1.129	
Percentiles	¼	2 (Unlikely)	
	½	3 (likely after 15 years)	
	¾	4 (likely before 15 years)	
Mann-Whitney U		Statistic	P-value
		-2.190	0.028
Wilcoxon (H0: median = 3)		Statistic	P-value
		-0.502	0.616
Kolmogorov-Smirnov		Statistic	P-value
		0.199	0.015
Shapiro-Wilk		Statistic	P-value
		0.857	0.003

		Unknown	Unlikely	Likely after 15 years	Likely before 15 years	Total
Good knowledge	Number of observations	5	4	3	3	15
	Frequency	33.3%	26.7%	20.0%	20.0%	100.0%
High Knowledge	Number of observations	0	1	4	4	9
	Frequency	0.0%	11.1%	44.4%	44.4%	100.0%

1.5.2 Please rank the following approaches from most likely to least likely to be successful in fixing or replacing the malfunctioning CFTR gene in the next 15 years

	Most likely (1)	2	3	Least likely (4)
CRISPR-Cas9	50,00%	0,00%	12,50%	37,50%
TALENs	16,70%	33,30%	50,00%	0,00%
Meganucleases	0,00%	16,70%	33,30%	50,00%
Zinc Finger Nucleases (ZFNs)	33,30%	50,00%	0,00%	16,70%

		CRISPR-Cas9	TALENs	Meganucleases	Zinc Finger Nucleases (ZFNs)
Mann-Whitney U	Statistic	-2.449	-1.000	-1.000	-2.000
	P-value	0.014	0.317	0.317	0.046

CRISPR-Cas9						
		1	2	3	4	Total
Good knowledge	Number of observations	0	0	0	3	3
	Frequency	0.0%	0.0%	0.0%	100.0%	100.0%
High Knowledge	Number of observations	4	0	1	0	5
	Frequency	80.0%	0.0%	20.0%	0.0%	100.0%

TALENs						
		1	2	3	4	Total
Good knowledge	Number of observations	0	2	0	0	2
	Frequency	0.0%	100.0%	0.0%	0.0%	100.0%
High Knowledge	Number of observations	1	0	3	0	4
	Frequency	25.0%	0.0%	75.0%	0.0%	100.0%

Meganucleases						
		1	2	3	4	Total
Good knowledge	Number of observations	0	0	2	0	2
	Frequency	0.0%	0.0%	100.0%	0.0%	100.0%
High Knowledge	Number of observations	0	1	0	3	4
	Frequency	0.0%	25.0%	0.0%	75.0%	100.0%

Zinc Finger Nucleases (ZFNs)						
		1	2	3	4	Total
Good knowledge	Number of observations	2	0	0	0	2

	Frequency	100.0%	0.0%	0.0%	0.0%	100.0%
High Knowledge	Number of observations	0	3	0	1	4
	Frequency	0.0%	75.0%	0.0%	25.0%	100.0%

2 Descriptive statistics of the results according to the chosen therapeutic option and the respondents' level of knowledge

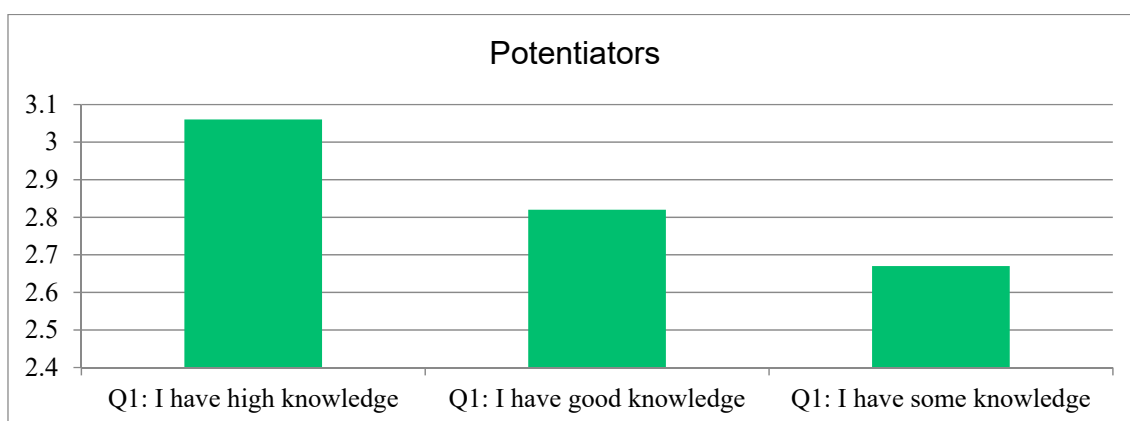
2.1 Respondents who selected CFTR modulator therapies as the most likely to be successful in treating CF in the next 15 years

2.1.1 Please rank the following approaches from most likely to least likely to be successful in treating cystic fibrosis in the next 15 years

Potentiators

	1	2	3	4	Total	Score
Q1: I have high knowledge	26.77% 34	58.27% 74	8.66% 11	6.30% 8	43.49% 127	3.06
Q1: I have good knowledge	24.17% 29	44.17% 53	20.83% 25	10.83% 13	41.10% 120	2.82
Q1: I have some knowledge	17.95% 7	43.59% 17	25.64% 10	12.82% 5	13.36% 39	2.67
Total	23.97% 70	49.32% 144	15.75% 46	8.90% 26	100.00% 292	

Answered 292



Correctors

	1	2	3	4	Total	Score
Q1: I have high knowledge	62.50% 80	23.44% 30	6.25% 8	7.81% 10	43.84% 128	3.41
Q1: I have good knowledge	51.24% 62	23.14% 28	12.40% 15	13.22% 16	41.44% 121	3.12
Q1: I have some knowledge	39.47% 15	18.42% 7	21.05% 8	21.05% 8	13.01% 38	2.76
Total	53.77% 157	22.26% 65	10.62% 31	11.64% 34	100.00% 292	

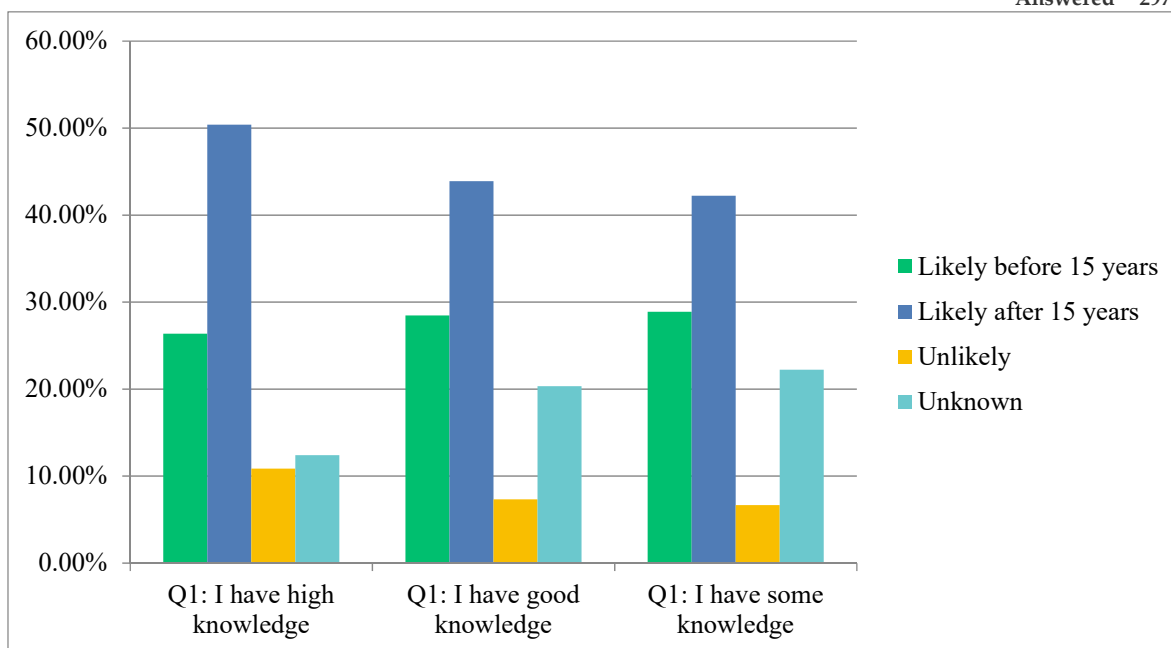
Answered 292



2.1.2 Fixing or replacing the CFTR gene will lead to a cure for cystic fibrosis

	Likely before 15 years		Likely after 15 years		Unlikely		Unknown		Total	
Q1: I have high knowledge	26.36%	34	50.39%	65	10.85%	14	12.40%	16	43.43%	129
Q1: I have good knowledge	28.46%	35	43.90%	54	7.32%	9	20.33%	25	41.41%	123
Q1: I have some knowledge	28.89%	13	42.22%	19	6.67%	3	22.22%	10	15.15%	45
Total	27.61%	82	46.46%	138	8.75%	26	17.17%	51	100.00%	297

Answered 297

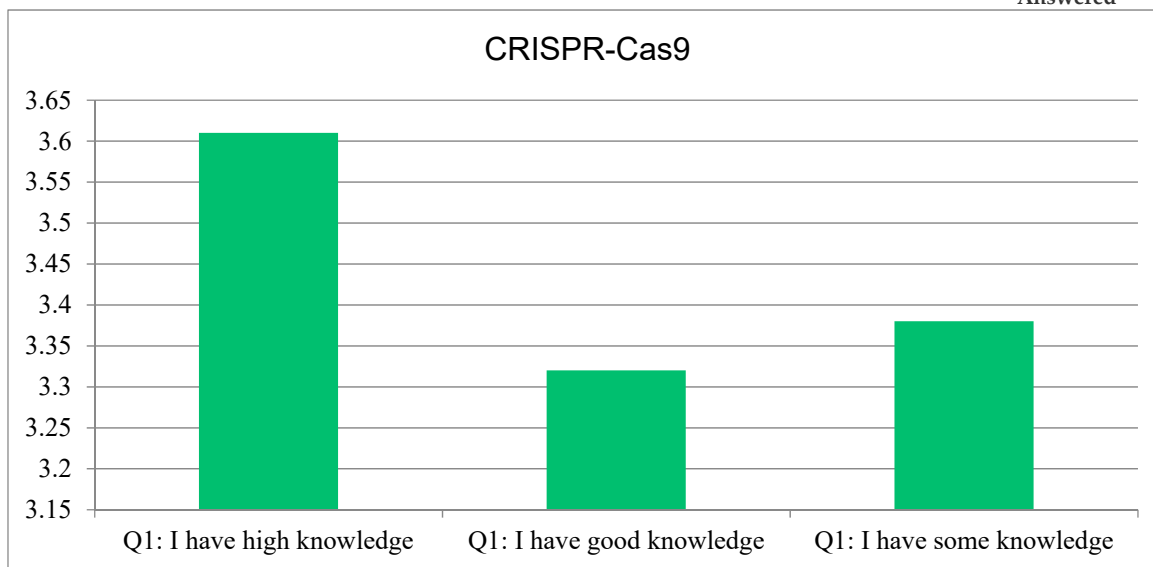


2.1.3 Please rank the following approaches from most likely to least likely to be successful in fixing or replacing the malfunctioning CFTR gene in the next 15 years

CRISPR-Cas9

	1		2		3		4		Total		Score	
Q1: I have high knowledge	78.31%	65	10.84%	9	4.82%	4	6.02%	5	45.86%	83	3.61	
Q1: I have good knowledge	66.20%	47	15.49%	11	2.82%	2	15.49%	11	39.23%	71	3.32	
Q1: I have some knowledge	70.83%	17	8.33%	2	8.33%	2	12.50%	3	13.26%	24	3.38	
Total	71.27%	129	12.15%	22	4.42%	8	10.50%	19	100.00%	181		

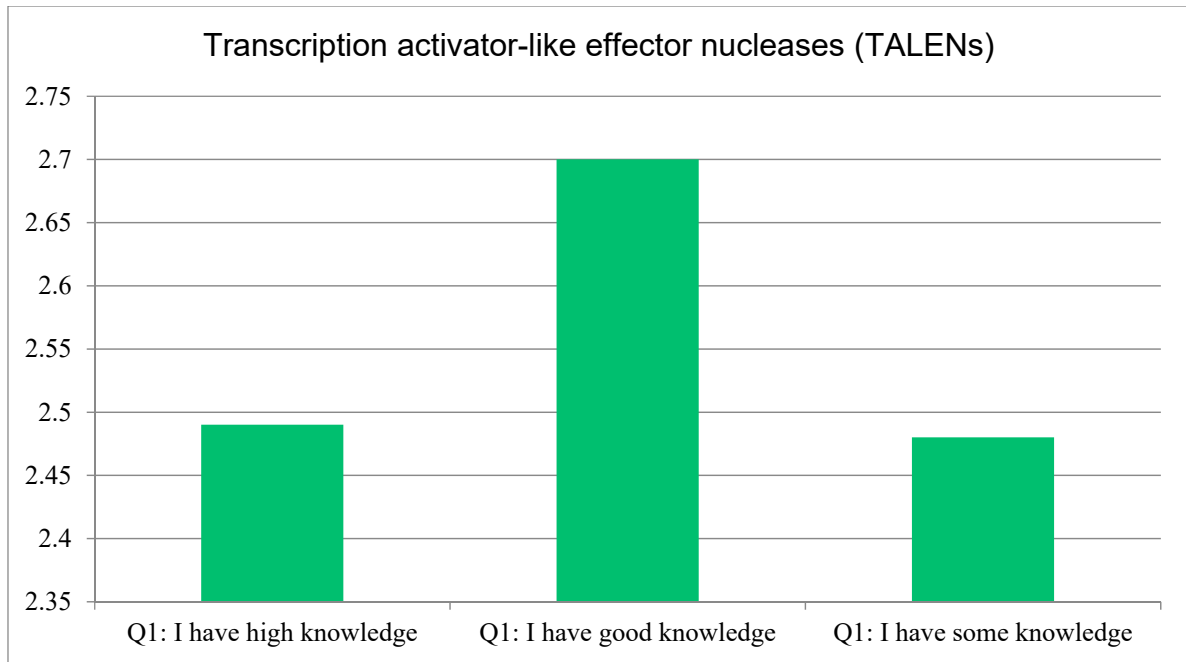
Answered 181



TALENs

	1		2		3		4		Total	Score	
Q1: I have high knowledge	12.20%	10	41.46%	34	29.27%	24	17.07%	14	45.30%	82	2.49
Q1: I have good knowledge	17.91%	12	46.27%	31	23.88%	16	11.94%	8	37.02%	67	2.7
Q1: I have some knowledge	19.05%	4	33.33%	7	23.81%	5	23.81%	5	11.60%	21	2.48
Total	14.36%	26	39.78%	72	24.86%	45	14.92%	27	100.00%	181	

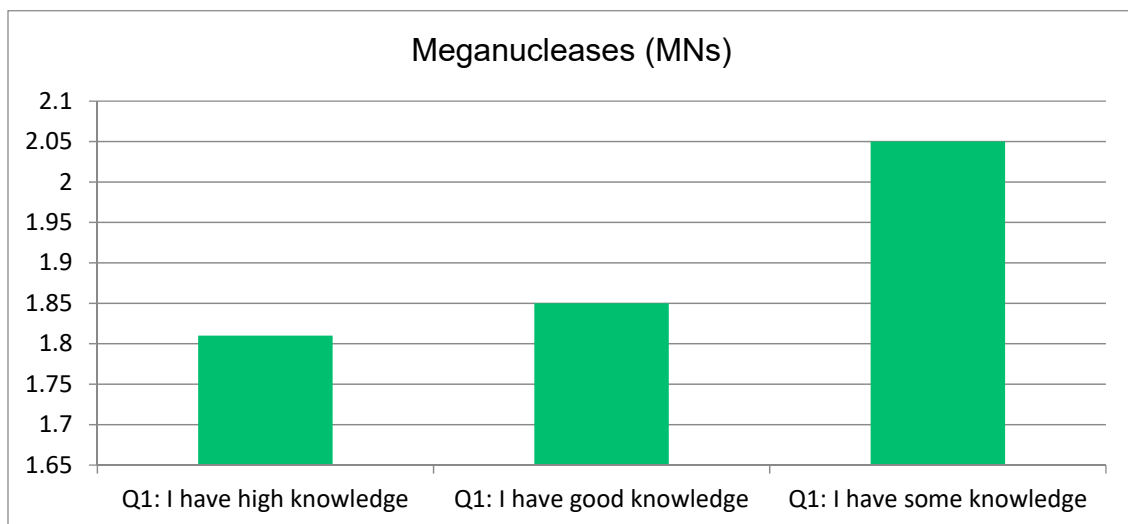
Answered 181



Meganucleases (MNs)

	1		2		3		4		Total	Score	
Q1: I have high knowledge	6.25%	5	13.75%	11	35.00%	28	45.00%	36	44.20%	80	1.81
Q1: I have good knowledge	10.29%	7	8.82%	6	36.76%	25	44.12%	30	37.57%	68	1.85
Q1: I have some knowledge	0.00%	0	31.82%	7	40.91%	9	27.27%	6	12.15%	22	2.05
Total	6.63%	12	13.26%	24	34.25%	62	39.78%	72	100.00%	181	

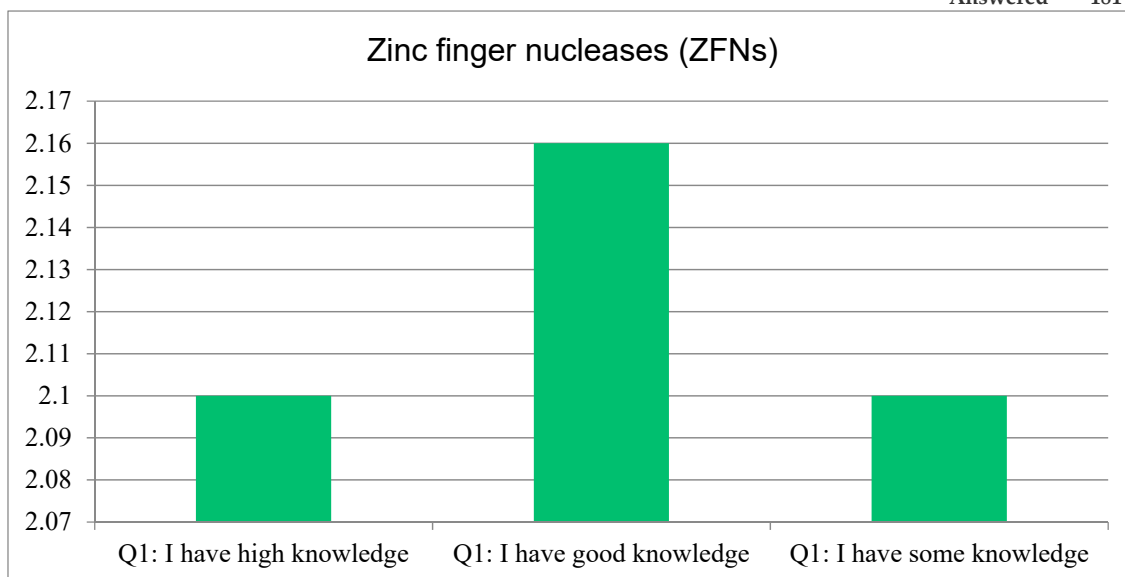
Answered 181



Zinc finger nucleases (ZFNs)

	1	2	3	4	Total	Score
Q1: I have high knowledge	3.70% 3	33.33% 27	32.10% 26	30.86% 25	44.75% 81	2.1
Q1: I have good knowledge	7.46% 5	28.36% 19	37.31% 25	26.87% 18	37.02% 67	2.16
Q1: I have some knowledge	9.52% 2	28.57% 6	23.81% 5	38.10% 8	11.60% 21	2.1
Total	5.52% 10	28.73% 52	30.94% 56	28.18% 51	100.00% 181	

Answered 181



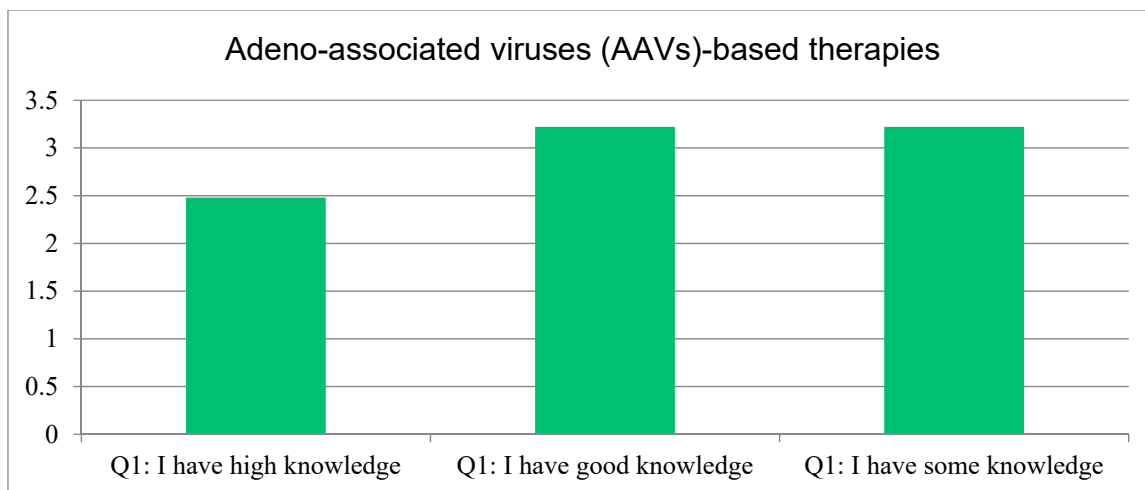
2.2 Respondents who selected genetic therapies as the most likely to be successful in treating CF in the next 15 years

2.2.1 Please rank the following therapies from most likely to least likely to be successful in treating CF in the next 15 years

AAVs-based therapies

	1	2	3	4	5	Total	Score
Q1: I have high knowledge	12.00% 3	20.00% 5	8.00% 2	24.00% 6	36.00% 9	27.17% 25	2.48
Q1: I have good knowledge	22.22% 8	13.89% 5	36.11% 13	19.44% 7	8.33% 3	39.13% 36	3.22
Q1: I have some knowledge	26.09% 6	26.09% 6	8.70% 2	21.74% 5	17.39% 4	25.00% 23	3.22
Total	18.48% 17	17.39% 16	18.48% 17	19.57% 18	17.39% 16	100.00% 92	

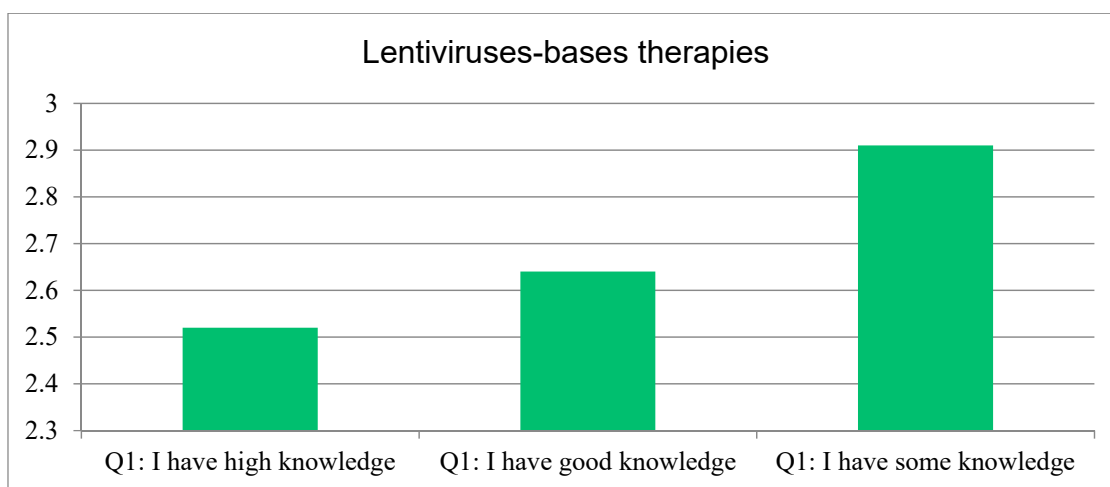
Answered 92



Lentiviruses-bases therapies

	1	2	3	4	5	Total	Score
Q1: I have high knowledge	11.11% 3	11.11% 3	18.52% 5	37.04% 10	22.22% 6	29.35%	27 2.52
Q1: I have good knowledge	8.33% 3	22.22% 8	13.89% 5	36.11% 13	19.44% 7	39.13%	36 2.64
Q1: I have some knowledge	22.73% 5	9.09% 2	18.18% 4	36.36% 8	13.64% 3	23.91%	22 2.91
Total	11.96% 11	14.13% 13	15.22% 14	33.70% 31	17.39% 16	100.00%	92

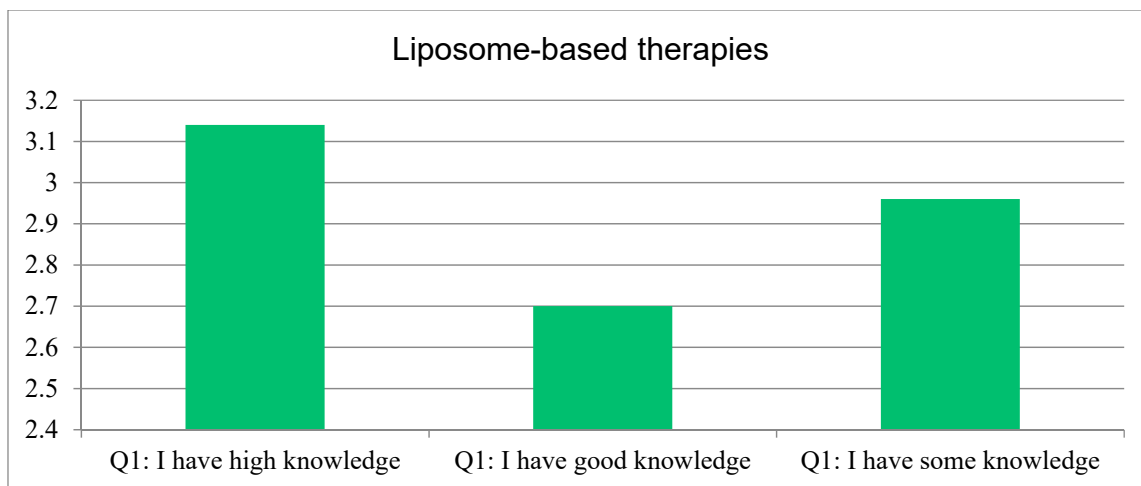
Answered 92



Liposome-based therapies

	1	2	3	4	5	Total	Score
Q1: I have high knowledge	28.57% 8	17.86% 5	21.43% 6	3.57% 1	28.57% 8	30.43%	28 3.14
Q1: I have good knowledge	13.51% 5	21.62% 8	18.92% 7	13.51% 5	32.43% 12	40.22%	37 2.7
Q1: I have some knowledge	13.04% 3	21.74% 5	30.43% 7	17.39% 4	17.39% 4	25.00%	23 2.96
Total	17.39% 16	19.57% 18	21.74% 20	10.87% 10	26.09% 24	100.00%	92

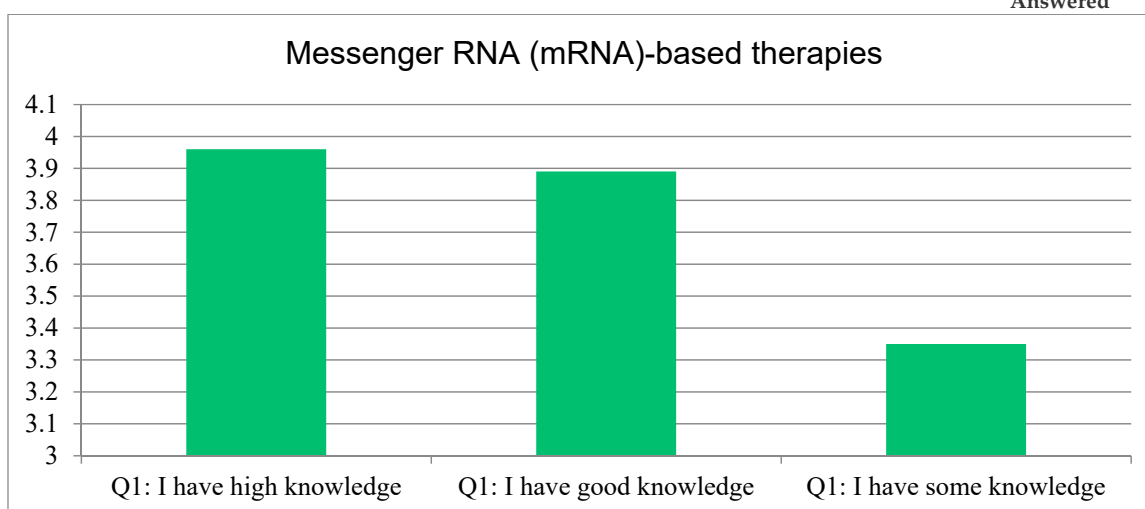
Answered 92



mRNA-based therapies

	1	2	3	4	5	Total	Score
Q1: I have high knowledge	40.74% 11	29.63% 8	18.52% 5	7.41% 2	3.70% 1	29.35%	27 3.96
Q1: I have good knowledge	44.74% 17	21.05% 8	18.42% 7	10.53% 4	5.26% 2	41.30%	38 3.89
Q1: I have some knowledge	34.62% 9	19.23% 5	11.54% 3	15.38% 4	19.23% 5	28.26%	26 3.35
Total	40.22% 37	22.83% 21	16.30% 15	10.87% 10	8.70% 8	100.00%	92

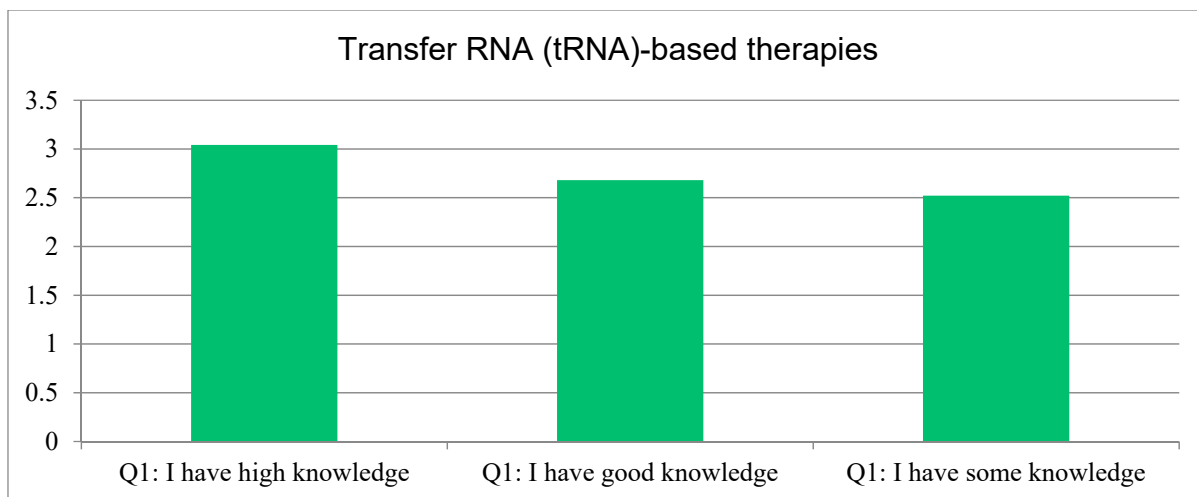
Answered 92



tRNA-based therapies

	1	2	3	4	5	Total	Score
Q1: I have high knowledge	11.54% 3	23.08% 6	30.77% 8	26.92% 7	7.69% 2	28.26%	26 3.04
Q1: I have good knowledge	13.16% 5	23.68% 9	13.16% 5	18.42% 7	31.58% 12	41.30%	38 2.68
Q1: I have some knowledge	4.76% 1	23.81% 5	28.57% 6	4.76% 1	38.10% 8	22.83%	21 2.52
Total	9.78% 9	21.74% 20	20.65% 19	16.30% 15	23.91% 22	100.00%	92

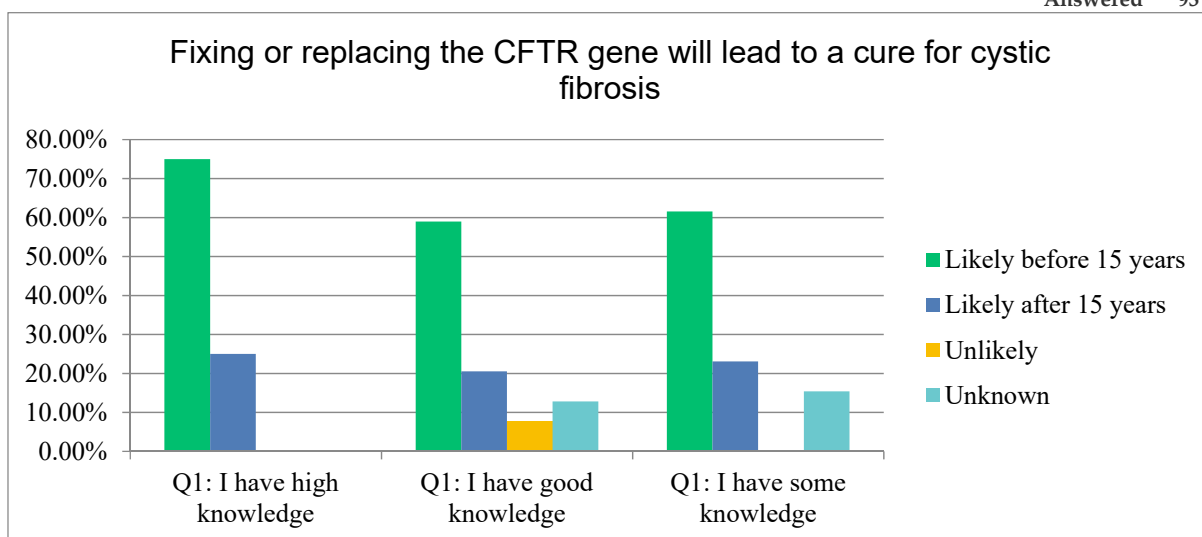
Answered 92



2.2.2 Fixing or replacing the CFTR gene will lead to a cure for cystic fibrosis

	Likely before 15 years		Likely after 15 years		Unlikely		Unknown		Total	
Q1: I have high knowledge	75.00%	21	25.00%	7	0.00%	0	0.00%	0	30.11%	28
Q1: I have good knowledge	58.97%	23	20.51%	8	7.69%	3	12.82%	5	41.94%	39
Q1: I have some knowledge	61.54%	16	23.08%	6	0.00%	0	15.38%	4	27.96%	26
Total	64.52%	60	22.58%	21	3.23%	3	9.68%	9	100.00%	93

Answered 93

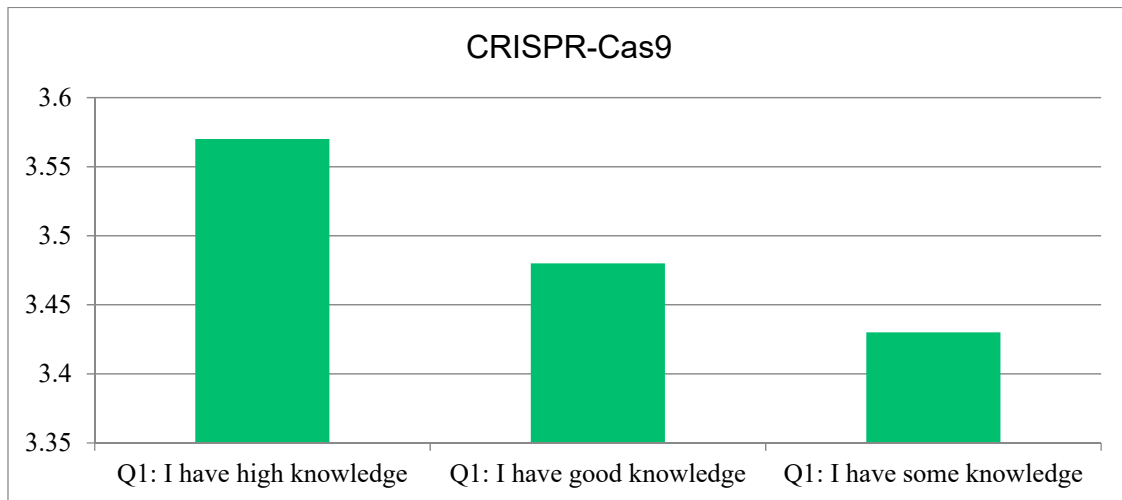


2.2.3 Please rank the following approaches from most likely to least likely to be successful in fixing or replacing the malfunctioning CFTR gene in the next 15 years

CRISPR-Cas9

	1	2	3	4	Total	Score
Q1: I have high knowledge	82.14% 23	3.57% 1	3.57% 1	10.71% 3	32.94%	28 3.57
Q1: I have good knowledge	75.76% 25	6.06% 2	9.09% 3	9.09% 3	38.82%	33 3.48
Q1: I have some knowledge	69.57% 16	13.04% 3	8.70% 2	8.70% 2	27.06%	23 3.43

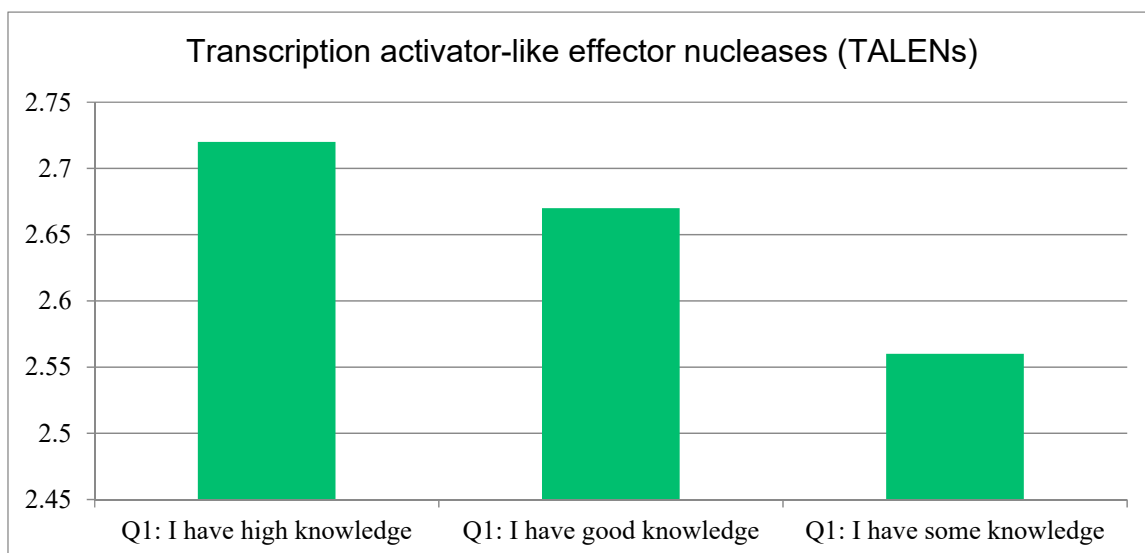
Total	75.29%	64	7.06%	6	7.06%	6	9.41%	8	100.00%	85
									Answered	85



TALENs

	1		2		3		4		Total	Score	
Q1: I have high knowledge	8.00%	2	64.00%	16	20.00%	5	8.00%	2	29.41%	25	2.72
Q1: I have good knowledge	16.67%	5	43.33%	13	30.00%	9	10.00%	3	35.29%	30	2.67
Q1: I have some knowledge	5.56%	1	61.11%	11	16.67%	3	16.67%	3	21.18%	18	2.56
Total	9.41%	8	47.06%	40	20.00%	17	9.41%	8	100.00%	85	

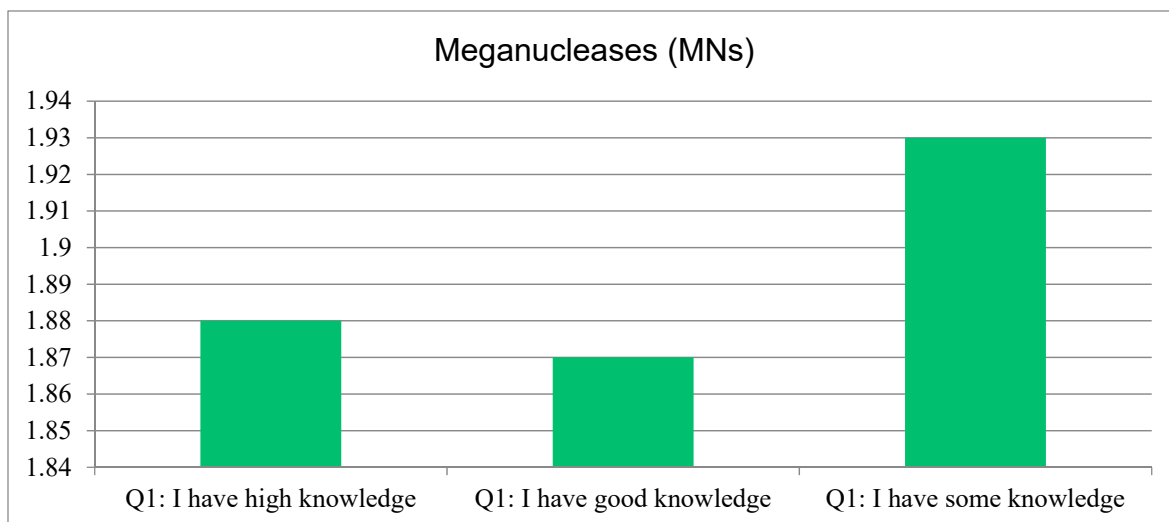
Answered 85



Meganucleases (MNs)

	1		2		3		4		Total	Score	
Q1: I have high knowledge	3.85%	1	19.23%	5	38.46%	10	38.46%	10	30.59%	26	1.88
Q1: I have good knowledge	6.67%	2	23.33%	7	20.00%	6	50.00%	15	35.29%	30	1.87
Q1: I have some knowledge	13.33%	2	13.33%	2	26.67%	4	46.67%	7	17.65%	15	1.93
Total	5.88%	5	16.47%	14	23.53%	20	37.65%	32	100.00%	85	

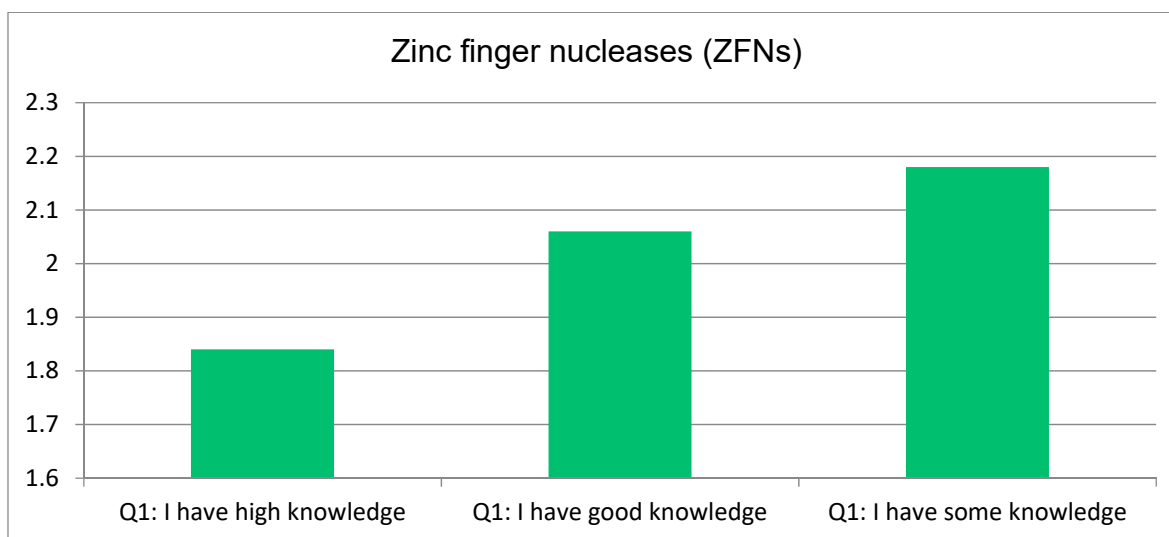
Answered 85



Zinc finger nucleases (ZFNs)

	1	2	3	4	Total	Score
Q1: I have high knowledge	8.00% 2	12.00% 3	36.00% 9	44.00% 11	29.41%	25 1.84
Q1: I have good knowledge	3.23% 1	29.03% 9	38.71% 12	29.03% 9	36.47%	31 2.06
Q1: I have some knowledge	11.76% 2	17.65% 3	47.06% 8	23.53% 4	20.00%	17 2.18
Total	5.88% 5	17.65% 15	34.12% 29	28.24% 24	100.00%	85

Answered 85

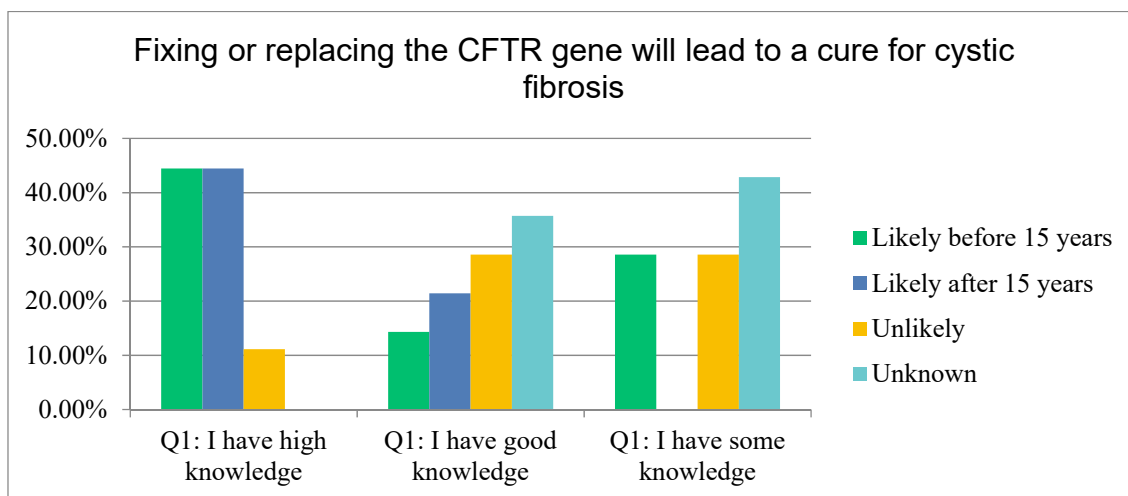


2.3 Respondents who selected 'other' option as the most likely to be successful in treating CF in the next 15 years

2.3.1 Fixing or replacing the CFTR gene will lead to a cure for cystic fibrosis

	Likely before 15 years		Likely after 15 years		Unlikely		Unknown		Total	
Q1: I have high knowledge	44.44%	4	44.44%	4	11.11%	1	0.00%	0	30.00%	9
Q1: I have good knowledge	14.29%	2	21.43%	3	28.57%	4	35.71%	5	46.67%	14
Q1: I have some knowledge	28.57%	2	0.00%	0	28.57%	2	42.86%	3	23.33%	7
Total	26.67%	8	23.33%	7	23.33%	7	26.67%	8	100.00%	30

Answered 30

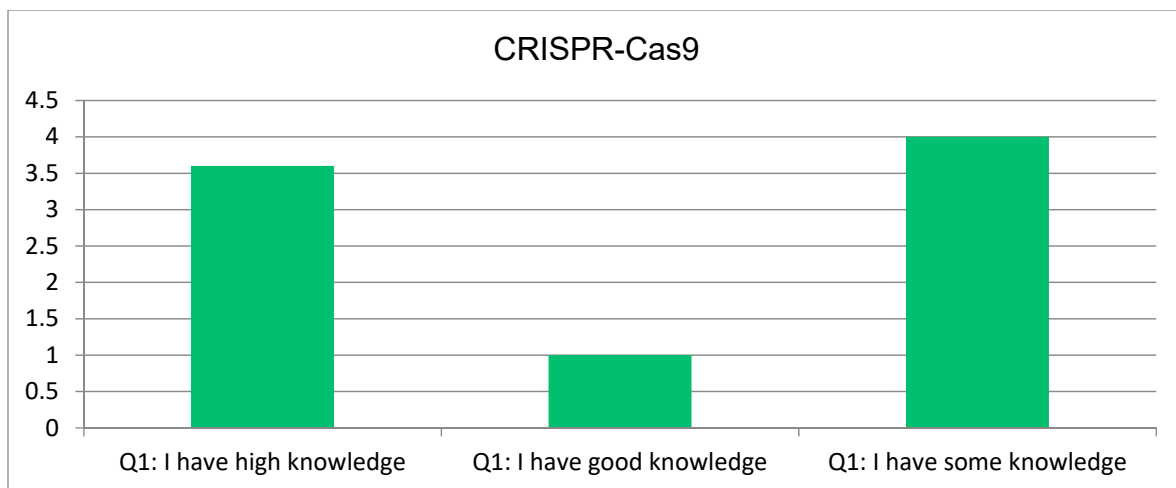


2.3.2 Please rank the following approaches from most likely to least likely to be successful in fixing or replacing the malfunctioning CFTR gene in the next 15 years

CRISPR-Cas9

	1		2		3		4		Total	Score
Q1: I have high knowledge	80.00%	4	0.00%	0	20.00%	1	0.00%	0	55.56%	5 3.6
Q1: I have good knowledge	0.00%	0	0.00%	0	0.00%	0	100.00%	3	33.33%	3 1
Q1: I have some knowledge	100.00%	1	0.00%	0	0.00%	0	0.00%	0	11.11%	1 4
Total	55.56%	5	0.00%	0	11.11%	1	33.33%	3	100.00%	9

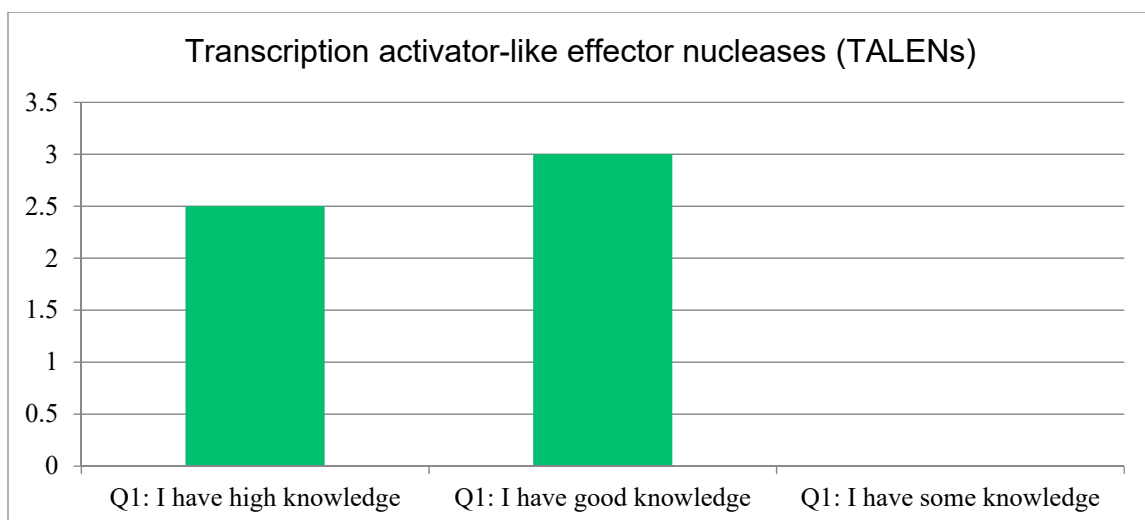
Answered 9



TALENs

	1		2		3		4		Total	Score
Q1: I have high knowledge	25.00%	1	0.00%	0	75.00%	3	0.00%	0	44.44%	4
Q1: I have good knowledge	0.00%	0	100.00%	2	0.00%	0	0.00%	0	22.22%	2
Q1: I have some knowledge	0.00%	0	0.00%	0	0.00%	0	0.00%	0	0.00%	0
Total	11.11%	1	22.22%	2	33.33%	3	0.00%	0	100.00%	9

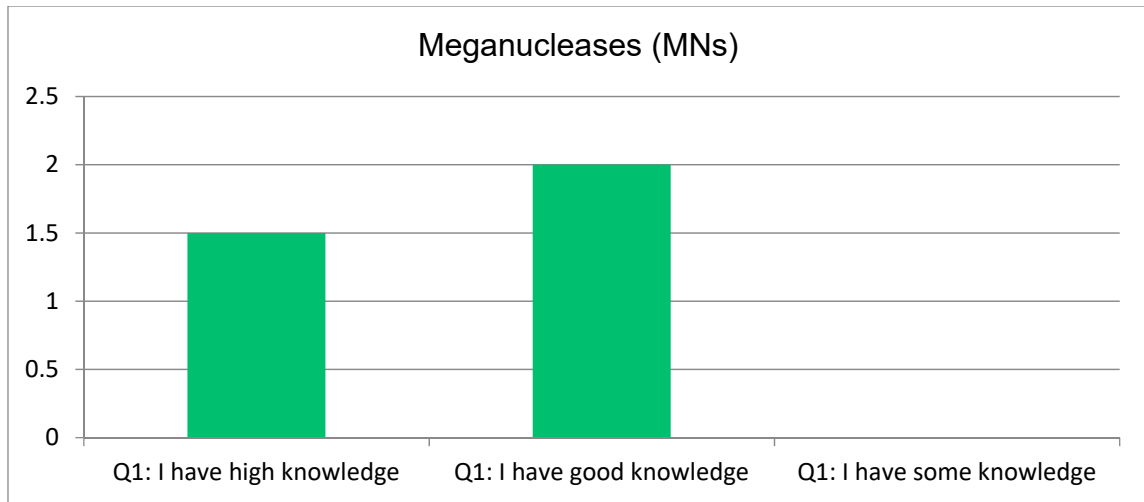
Answered 9



Meganucleases (MNs)

	1		2		3		4		Total	Score
Q1: I have high knowledge	0.00%	0	25.00%	1	0.00%	0	75.00%	3	44.44%	4
Q1: I have good knowledge	0.00%	0	0.00%	0	100.00%	2	0.00%	0	22.22%	2
Q1: I have some knowledge	0.00%	0	0.00%	0	0.00%	0	0.00%	0	0.00%	0
Total	0.00%	0	11.11%	1	22.22%	2	33.33%	3	100.00%	9

Answered 9



Zinc finger nucleases (ZFNs)

	1		2		3		4		Total	Score	
Q1: I have high knowledge	0.00%	0	75.00%	3	0.00%	0	25.00%	1	44.44%	4	2.5
Q1: I have good knowledge	100.00%	2	0.00%	0	0.00%	0	0.00%	0	22.22%	2	4
Q1: I have some knowledge	0.00%	0	100.00%	1	0.00%	0	0.00%	0	11.11%	1	3
Total	22.22%	2	44.44%	4	0.00%	0	11.11%	1	100.00%	9	
Answered										9	

