

**Assessing Patient Preferences in Rare Diseases: Direct Preference Elicitation in the
Rare Chronic Kidney Disease, Immunoglobulin A Nephropathy**

**Assessing Patient Preferences in Rare Diseases: Direct Preference Elicitation in
Immunoglobulin A Nephropathy**

Authors: Kevin Marsh^{1*}; Kerrie-Anne Ho²; Rachel Lo¹, Nancy Zaour³;

Aneesh Thomas George⁴; Nigel S Cook³

¹Evidera, London, UK; ²UCB Pharma, Slough, UK; ³Novartis Pharma AG, Basel, Switzerland,

⁴Novartis Healthcare Pvt. Ltd, Hyderabad, India

*Corresponding Author:

Kevin Marsh

Evidera, London, UK

Ph: +44 (0) 208 576 5025

Email: Kevin.Marsh@evidera.com

SUPPLEMENTARY MATERIAL – SUBGROUP ANALYSES

	Univariable analyses^{^†}					
Variables	Risk of infections	Risk of other side effects	Ability to do usual activities	Emotional burden	Number of vaccinations	[Ln Phi][±]
Country						
US	Reference					
China	0.323	0.509*	0.375	-0.139	0.099	0.294
	(0.257)	(0.256)	(0.254)	(0.302)	(0.339)	(0.197)
	[-0.181 - 0.828]	[0.007 - 1.012]	[-0.124 - 0.873]	[-0.731 - 0.452]	[-0.565 - 0.763]	[-0.091 - 0.679]
Age						
<50y	Reference					
50y and above	-0.608	-0.629	-0.635	-0.352	-0.049	-0.781***
	(0.335)	(0.337)	(0.333)	(0.366)	(0.400)	(0.218)
	[-1.265 - 0.049]	[-1.290 - 0.033]	[-1.288 - 0.018]	[-1.070 - 0.366]	[-0.833 - 0.735]	[-1.208 - -0.355]

Eliciting Patient Preferences for Treatment for Rare Diseases

	Univariable analyses [†]					
Variables	Risk of infections	Risk of other side effects	Ability to do usual activities	Emotional burden	Number of vaccinations	[Ln Phi] [±]
Employment						
In employment	Reference					
Not in employment	0.239	0.432	0.591*	0.479	-0.448	0.859**
	(0.290)	(0.283)	(0.272)	(0.321)	(0.441)	(0.274)
	[-0.330 - 0.807]	[-0.123 - 0.986]	[0.057 - 1.126]	[-0.151 - 1.109]	[-1.313 - 0.417]	[0.323 - 1.395]
Education						
With college/university degree	Reference					
No college/university degree	0.308	0.507*	0.103	-0.266	0.260	0.442*
	(0.253)	(0.252)	(0.254)	(0.302)	(0.332)	(0.200)
	[-0.188 - 0.805]	[0.014 - 1.000]	[-0.395 - 0.601]	[-0.857 - 0.326]	[-0.391 - 0.912]	[0.051 - 0.833]

Eliciting Patient Preferences for Treatment for Rare Diseases

	Univariable analyses [†]					
Variables	Risk of infections	Risk of other side effects	Ability to do usual activities	Emotional burden	Number of vaccinations	[Ln Phi] [±]
Time since diagnosis						
<10y since diagnosis	Reference					
10y and more since diagnosis	-0.467	-0.299	-0.525	-0.524	0.270	-0.702***
	(0.278)	(0.276)	(0.277)	(0.314)	(0.344)	(0.195)
	[-1.012 - 0.078]	[-0.841 - 0.243]	[-1.067 - 0.017]	[-1.140 - 0.092]	[-0.405 - 0.945]	[-1.084 - -0.319]
Corticosteroid treatment status						
Not currently taking corticosteroids	Reference					
Currently taking corticosteroids	0.201	0.076	0.389	-0.054	0.309	0.519
	(0.314)	(0.322)	(0.301)	(0.378)	(0.411)	(0.267)
	[-0.414 - 0.816]	[-0.555 - 0.708]	[-0.202 - 0.980]	[-0.795 - 0.686]	[-0.497 - 1.115]	[-0.005 - 1.043]

Eliciting Patient Preferences for Treatment for Rare Diseases

	Univariable analyses[†]					
Variables	Risk of infections	Risk of other side effects	Ability to do usual activities	Emotional burden	Number of vaccinations	[Ln Phi][±]
Immunosuppressant treatment status						
Not currently taking immunosuppressants	Reference					
Currently taking immunosuppressants	-0.165	-0.749	0.098	-0.062	-0.392	0.396
	(0.374)	(0.427)	(0.351)	(0.425)	(0.527)	(0.322)
	[-0.898 - 0.568]	[-1.586 - 0.089]	[-0.589 - 0.786]	[-0.895 - 0.771]	[-1.424 - 0.641]	[-0.236 - 1.028]
Level of emotional burden						
Not at all/somewhat	Reference					
Very much emotionally burdened	-0.033	0.654	0.147	0.054	0.658	0.431
	(0.402)	(0.356)	(0.384)	(0.454)	(0.468)	(0.317)
	[-0.820 - 0.755]	[-0.043 - 1.351]	[-0.606 - 0.899]	[-0.836 - 0.943]	[-0.258 - 1.575]	[-0.190 - 1.052]

Eliciting Patient Preferences for Treatment for Rare Diseases

	Univariable analyses [†]					
Variables	Risk of infections	Risk of other side effects	Ability to do usual activities	Emotional burden	Number of vaccinations	[Ln Phi] [±]
Ability to do usual activity						
Not at all/ somewhat	Reference					
Very much able to do usual activity	-0.359	-0.251	0.047	-0.062	0.187	-0.086
	(0.264)	(0.268)	(0.269)	(0.307)	(0.352)	(0.199)
	[-0.877 - 0.159]	[-0.775 - 0.274]	[-0.480 - 0.575]	[-0.664 - 0.540]	[-0.504 - 0.877]	[-0.476 - 0.305]
KDCS score						
<60	Reference					
>60	-0.089	0.207	0.153	-0.184	-0.737*	0.771***
	(0.248)	(0.248)	(0.245)	(0.283)	(0.329)	(0.191)
	[-0.574 - 0.397]	[-0.280 - 0.693]	[-0.327 - 0.633]	[-0.740 - 0.372]	[-1.382 - -0.091]	[0.395 - 1.146]
EQ5D score						
	-0.601	0.289	-0.011	0.135	-0.629	0.083
	(0.659)	(0.798)	(0.699)	(0.809)	(0.813)	(0.676)
	[-1.893 - 0.691]	[-1.275 - 1.852]	[-1.381 - 1.360]	[-1.450 - 1.720]	[-2.223 - 0.965]	[-1.242 - 1.407]

Standard errors in parentheses

Eliciting Patient Preferences for Treatment for Rare Diseases

95% Confidence interval in brackets

*** $p < 0.001$, ** $p < 0.01$, * $p < 0.05$

^Loglikelihood of univariable models are: KDCS score= 258.6, EQ5D score =249.8, Ability to do usual activity= 250.8, Level of emotional burden = 251.8, Experience with immunosuppressant=251.2, Experience with corticosteroids=251.6, Time since diagnosis=261.0, Education=254.7, Employment=255.3, Age=262.4, Country=253.1

† The coefficients represent the effect of the observable characteristics on the relative importance of the treatment attributes relative to Likelihood of ESRD; a positive coefficient represents increasing importance while a negative coefficient represents decreasing importance.

± Ln Phi is the precision parameter that represents the shape of the Dirichlet distribution