**Supplementary File 1: online survey sent to SMC committee members**

**Patient and Clinician Engagement (PACE) Evaluation**

**In 2016 the SMC team conducted a thematic analysis of Patient and Clinician Engagement (PACE) statements to identify key themes that were important to patients and their families/carers. We are now looking at the relative importance of these themes to SMC committee members when making decisions on PACE medicines i.e. orphan, end of life and / or ultra-orphan medicines.**

**How long have you served on the SMC committee?**

|  |  |
| --- | --- |
| https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | Less than 1 year |
| https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | 1-3 years |
| https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | Longer than 3 years |

**The 2016 analysis identified three prominent themes relating to the potential value of a new medicine for patients and their families/carers: quality of life; life expectancy and unmet need. When you are making a decision about a PACE medicine in general, how important do you consider information relating to each theme?**

|  |  |  |  |  |
| --- | --- | --- | --- | --- |
|  | Very important | Important | Of lesser importance | Not important |
| Impact on quality of Life | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   |
| Impact on life expectancy | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   |
| Addresses unmet need | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   |

**Would you rate the importance of these themes differently depending on whether it is an orphan, end of life or ultra-orphan medicine? Please explain your response.**

|  |  |
| --- | --- |
| https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | Yes |
| https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | No |
| https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | Maybe |

Please explain

|  |
| --- |
|   |

**Analysis of the ‘quality of life’ theme identified 8 sub-themes. When you are making a decision about a PACE medicine in general, how important do you consider information relating to each of these sub-themes?**

|  |  |  |  |  |
| --- | --- | --- | --- | --- |
|  | Very important | Important | Of lesser importance | Not important |
| Ability to take part in normal life (including work, education, social and family activities) | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   |
| Convenience of administration | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   |
| Health benefits (improved physical health and better symptom control) | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   |
| Impact on patient’s family or carer | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   |
| Opportunity for treatment choice | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   |
| Providing hope for the future | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   |
| Psychological benefit (for example improved emotional well-being, reduced stress, reduced anxiety and improved self-esteem) | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   |
| Tolerability (relating to side-effect profile) | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   |

**Would you rate the importance of these themes differently depending on whether it is an orphan, end of life or ultra-orphan medicine? Please explain your response.**

|  |  |
| --- | --- |
| https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | Yes |
| https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | No |
| https://app.smartsurvey.co.uk/images/ico/print/checkbox.png   | Maybe |

Please explain

|  |
| --- |
|   |

**Please state whether there are other themes or elements of the PACE statement that are important in your decision making.**

|  |
| --- |
|   |