**Respondent characteristics**

Haut du formulaire

Please indicate which of the following **best characterises the organisation you represent**:

1. Please indicate which of the following **best characterises the organisation you represent**:

Pharmaceutical or biotechnology company **with** **own R&D activity**

Pharmaceutical or biotechnology company **without any own R&D activity**

**Academic institution or research institute**

**National professional association** for the pharmaceutical or biotechnology industry

**European professional association** for the pharmaceutical or biotechnology industry

Other (please specify)

\*2. Please provide **the name of your organisation**

3. **How many employees** does your organisation have (in full time equivalents)?

**More than 500**

**Between 251 and 500**

**Between 51 and 250**

**Between 10 and 50**

**Fewer than 10**

4. Is your organisation currently listed on the**EMA SME Register**?

**Yes**

**No**

5. Is your organisation currently, or has it ever been, **a sponsor for any EU designated orphan medicinal product**?

**Yes**

**No**

6. **For how many products** (not limited to EU designated orphan medicinal products) has your organisation **obtained a European marketing authorisation**?

**None**

**Between 1 and 3**

**Between 4 and 10**

**More than 10**

**R&D activities: General**

Haut du formulaire

\*7. Does your organisation currently conduct **any R&D activities** to support development of medicinal products?

**Yes**

**No**

Bas du formulaire

**R&D activities: Development of medicines for rare diseases**

Haut du formulaire

8. In what **therapeutic area(s)**has your organisation ever conducted **R&D which could result in an application for an EU orphan designation**, irrespective of whether these activities have been discontinued or whether marketing authorisation for a product has been obtained?

Alimentary tract and metabolism (A)

Blood and blood forming organs (B)

Cardiovascular system (C)

Dermatologicals (D)

Genito-urinary system and sex hormones (G)

Systemic hormonal preparations, excluding sex hormones and insulins (H)

Anti-infectives for systemic use (J)

Antineoplastic and immunomodulating agents (L)

Musculo-skeletal system (M)

Nervous system (N)

Antiparasitic products, insecticides and repellents (P)

Respiratory system (R)

Sensory organs (S)

Various (V)

None

\*9. What are/were the **main reasons** for your organisation**to be active** in these particular therapeutic areas? (**Please provide max. 3 answers).**

Building on **existing R&D programmes and/or scientific expertise** in these areas

**Asset with properties potentially applicable** in other diseases or indications

**Obtainment of an R&D portfolio** (e.g. through acquisition, merger or in-licensing)

**Strong scientific, regulatory and commercial expertise** in this area

Availability of**scientific leads for further R&D**

Addressing areas of **greatest unmet need**

Expectation of being **first on the market**

**Personal commitment of company leadership** (e.g. due to family members suffering from a particular disease)

**Do not know**

Please, provide a brief explanation.

\*10. What are/were the **main reasons** for your organisation to **not be active** in development of orphan medicines in other therapeutic areas? **(Please provide max. 3 answers).**

**Insufficient fit** with overall company focus and R&D pipeline

**Lack of scientific expertise** in these areas

**Lack of scientific leads** for further R&D in these areas

**Lack of research and/or production facilities** to support R&D in these areas

Expectation of **insufficient access** to patients at a commercially viable price

**Insufficient ability** to generate data to support marketing authorisation

**Do not know**

Please, provide a brief explanation

11. Does your organisation have, or has had, **more than one research programme** within the same therapeutic area focused on product development for treatment of a rare disease?

**Yes**

**No**

**Not applicable**

**R&D: Development of EU designated orphan medicinal products**

Haut du formulaire

12. Please indicate for how many products your organisation has ever...

*Note: Products that received more than one EU orphan designation should be counted only once here.*

**Applied** for EU orphan designation

**Received** **EU orphan designation** (i.e. application granted)

**Withdrawn** EU orphan designation prior to marketing authorisation

**Received EU marketing authorisation** **with confirmation** of orphan designation

**Received EU marketing authorisation but was denied confirmation** of orphan designation

13. How many EU designated orphan medicines does your organisation **currently have** where **development is ongoing** and **for which no marketing authorisation has yet been granted**?

*Note: please, provide the number as reported on the sponsor’s annual report on a designated orphan medicinal product to the EMA.*

14. **For how many** EU designated orphan medicines has your company **discontinued development**?

15. What was/were the main **reason(s) for discontinuing development**of EU designated orphan medicines?

Lack of efficacy **data**

**Safety issues** identified

Change in**R&D priorities**

**Another organisation** obtained orphan market exclusivity for a product with a similar EU orphan designation

**Insufficient expectations** of significant benefit over existing treatment options (pharmacological or non-pharmacological)

Existence of **other medicines** that are already being used for the target indication (e.g. through off-label usage)

Costs and/or complexities of **clinical trials** (e.g. difficulty of finding sufficient trial participants)

**Not applicable** (no development discontinued)

Other (please specify)

**R&D: paediatric medicines**

Haut du formulaire

\*16. Has your organisation ever developed any EU designated orphan medicines that were**intended primarily for use in paediatric patients**, regardless of whether these medicines obtained marketing authorisation?

**Yes**

**No**

**Do not know**

Haut du formulaire

\*17. What were the **main reasons to engage** in development of EU designated orphan medicines for treatment of **primarily paediatric patients**? (Multiple answers possible).

Alignment with other **own R&D activities**

Addressing areas of**greatest unmet need**

**Experience and expertise** with development of paediatric medicines

**Product already under development** with likely significant benefit in paediatric populations

Other (please specify)

Bas du formulaire

**Impact of the EU Orphan Regulation on innovator organisations**

Haut du formulaire

18. What do you consider the **biggest barriers** to the development of medicines for treatment of rare diseases?

Potential low **return on investment**

**Financing**

**Scientific**

**Regulatory**

Other (please specify)

19. Please indicate the **general importance** of each of the **incentives** offered under the EU Orphan Regulation to your organisation for overcoming the aforementioned barriers, **on a scale of 1 to 4**. (with **1 = not important at all, 4 = highly important**. If this incentive is **not applicable** to your organisation, **please indicate 0**).



20. Please indicate in which of the following ways the EU Orphan Regulation has **influenced your organisation’s R&D activities**. **(Multiple answers possible).**

Increased overall **R&D investment**

Creation of **scientific/therapeutic expertise**

Expanded the **product pipeline** of medicines for treatment of rare diseases

**Reallocated resources** to new therapeutic areas or conditions with focus on rare disease

**Reallocated R&D activities** to other **jurisdictions** (e.g. from US to Europe)

Increased **interactions with other organisations** active in R&D for rare diseases

**None**

**Do not know**

Other (please specify)

21. Please, provide a**brief explanation** of your answer above.

\*22. In which of the following ways, if any, has the EU Orphan Regulation **influenced your organisation**? **(Max. 3 answers).**

Increased**ability to attract investors**

Increased the **company value**

Increased **interactions with patient organizations** to inform and improve drug development

Contributed to **development of new business models**

Increased **engagement with patients** and/or new methods for patient engagement

Increased **incentive to invest** in repurposing existing treatments for orphan indications

Increased **expertise in new R&D techniques and technologies**

**None**

**Do not know**

Other (please specify)

23. Please, provide a **brief explanation** of your answer above.

\*24. What**challenges**, if any, has the EU Orphan Regulation created for your organisation? **(Max. 3 answers).**

**Increased competition** from other organisations in therapeutic areas in which the organisation was already active **prior to introduction of the EU Orphan Regulation**

Increased competition from other organisations in therapeutic areas where the organisation **became active after introduction of the EU Orphan Regulation**

Increased **administrative burden**

Increased **development timelines** resulting from regulatory processes for orphan designation

**Do not know / Not applicable**

**None**

Other (please specify)

25. What **additional measures** would be needed to further incentivise R&D of orphan medicines?

**Other financial incentives** (e.g. R&D tax breaks, R&D grants)

Clarity on **market access criteria**

Agreed **evidence standards** to support access (e.g. conditional approval, accelerated access)

**Support for patient registries** and **post-market data collection** and **surveillance**

Other (please specify)

26. Please, briefly explain your answer below

**Role of orphan designation and market exclusivity on competition**

Haut du formulaire

Bas du formulaire

27. Has **the fact, or likelihood,** of another organisation **obtaining an initial EU orphan designation** (i.e. prior to marketing authorisation) in the EU for their product ever **influenced your organisation’s decision** to initiate or continue R&D for a product covering the same indication(s)?

**Yes:** **No new R&D** was initiated for this specific indication

**Yes:** **Ongoing R&D** in this area was **delayed or stopped**

**Yes:** **Ongoing R&D** in this area was **refocused**

**No:** **Ongoing R&D was unaffected**

**Not applicable**

**Do not know**

Please, provide a brief explanation.

28. Has **the fact, or likelihood,** of another organisation **obtaining marketing authorisation** for an EU designated orphan medicine, resulting in granting of the orphan market exclusivity, ever i**nfluenced your organisation’s decision to initiate or continue R&D for a product covering the same indication(s)**?

**Yes: No new R&D** was initiated for this specific indication

**Yes: Ongoing R&D** in this area was**delayed or stopped**

**Yes: Ongoing R&D** in this area was**refocused**

**No: Ongoing R&D was unaffected**

**Not applicable**

**Do not know**

Please, provide a brief explanation.

**R&D investments**

Haut du formulaire

29. Please estimate the **average annual R&D expenditure (rounded to the nearest € million)** of your organisation **over the past five years** (all products or conditions at any stage of development, including basic research).**If you have no reliable information on this or prefer not to say, you may leave blank. You may clarify your answer and/or indicate how these estimates were determined.**

30. Please estimate the **average annual R&D expenditure (rounded to the nearest € million)** of your organisation **over the past five years**, on products which had potential application for the treatment of rare diseases (at any stage of development, including basic research),**irrespective of whether they received marketing authorisation or not**. **If you have no reliable information on this or prefer not to say, you may leave blank. You may clarify your answer and/or indicate how these estimates were determined.**

31. Please estimate the **total average R&D costs per product** of your organisation on EU designated orphan medicines that **obtained marketing authorisation** on the legal basis of Article 8(3) of Directive 2001/83/EC, in each of the following R&D stages. **If you have no reliable information on this or prefer not to say, you may leave blank. If no R&D is performed in a particular stage, please indicate the amount “€ 0 million”.**

**Basic research**: (€ \_\_ million)

**Preclinical R&D phases**: (€ \_\_ million)

**Combined clinical phases**: (€ \_\_ million)

**Not applicable**

32. **If your organisation has successfully developed both orphan and non-orphan medicines**, which of the following best describes the **average R&D investments per product** that were made for these? **If you have no reliable information on this or prefer not to say, you may leave blank.**

R&D costs for orphan medicines**exceed** those for non-orphan medicines **by a factor of 2 or more**

R&D costs for orphan medicines**exceed** those for non-orphan medicines **by a factor of less than 2**

R&D costs for both types of medicines **are comparable**

R&D costs for orphan medicines are **lower** than those for non-orphan medicines **by a factor of 2 or more**

R&D costs for orphan medicines are **lower** than those for non-orphan medicines**by a factor of less than 2**

**Do not know**

Please, provide a brief explanation.

33. If your organisation has obtained any **marketing authorisations** for a EU designated orphan medicine for a well-known compound (through a well-established medicinal use or hybrid application), please estimate the **average costs** that were associated with preparing the data dossier to support these applications.

**Do not know**

**Not applicable** (no such marketing authorisation)

**€ \_\_ million**

34. Please, provide a **brief explanation** of your answer above.

35. If your organisation has developed any orphan medicines with **more than one EU orphan designation** (which was confirmed upon authorisation), please estimate the **average additional R&D costs** **for subsequent authorised indications.**

**Do not know**

**Not applicable** (no authorised product with multiple EU orphan designations)

**€ \_\_ million**

36. Please, provide a **brief explanation** of your answer above.

\*37. Has your organisation ever developed a product for which it was required to demonstrate **clinical superiority over another product with an EU orphan designation that was under active orphan market exclusivity**? If 'Yes', please estimate the **additional costs** of development associated with demonstrating clinical superiority.

**Do not know**

**No**

**Not applicable**

**Yes, (€ \_\_\_ million).**

**Market presence and access**

Haut du formulaire

38. Please rate the main **market factors** that influence your organisation’s decision if, and when to bring authorised orphan medicines to market in particular EU countries **on a scale of 1 to 4 (1 = not important at all, 4 = extremely important).**

If you chose "**Other**" (please specify in the box below)

39. Please, provide a **brief explanation** of your answer above.

40. Please rate the **main national healthcare system factors** that influence your organisation’s decision **if, and when to bring authorised orphan medicines to market** in particular EU countries **on a scale of 1 to 4 (1 = not important at all, 4 = extremely important).**

If you chose "**Other**", please specify in the box below.

41. Please, provide a **brief explanation** of your answer above.

Bas du formulaire

Bas du formulaire

**Efficiency and effectiveness of EMA procedures**

Haut du formulaire

42. How clear are the **criteria for application** for the initial orphan designation for applicants?

**Very poor**

**Poor**

**Acceptable**

**Good**

**Very good**

**No opinion**

Please, provide a brief explanation.

43. How **predictable are the outcomes** of the application process for orphan designation?

**Very poor**

**Poor**

**Acceptable**

**Good**

**Very good**

**No opinion**

Please, provide a brief explanation.

44. How **transparent** are the **application processes** for orphan designation?

**Very poor**

**Poor**

**Acceptable**

**Good**

**Very good**

**No opinion**

Please, provide a brief explanation.

\*45. Has your organisation ever received **scientific advice and/or protocol assistance** from EMA for the development of orphan medicines?

**No, never**

**Do not know**

**Yes**, please specify how many times in total

Bas du formulaire46. Please **rate the efficiency of the EMA procedures** for initial application and confirmation to maintain EU orphan designation on each of the following dimensions.

Please, provide a brief explanation.

47. How would you rate the **appropriateness of the evidence** requested by EMA, in terms of the **quantity and type of information required**, to support the initial application for an orphan designation?

**Very poor**

**Poor**

**Acceptable**

**Good**

**Very good**

**No opinion / do not know**

Please provide a brief explanation.

48. How would you rate the **appropriateness of the standard of evidence**, in terms of the **quantity and type of information required**, to confirm the orphan designation at the time of marketing authorisation?

**Very poor**

**Poor**

**Acceptable**

**Good**

**Very good**

**No opinion / do not know**

Please provide a brief explanation.

49. How would you **rate the coordination between the different EMA committees** (i.e. COMP, PDCO (for paediatric investigations), CHMP (for all marketing authorisations), CAT (for advanced therapies)) involved in the assessment of EU designated orphan medicines of your organisation on each of the following dimensions?

Please, provide a brief explanation.

**End of the survey!**

Haut du formulaire

50. If you have **any relevant documentation** you wish to share to further clarify your responses, **please upload this here or send it to the email address orphan-regulation@technopolis-group.com**.

Choose File

 No file chosen

51. If you have **any further comments** about the EU Orphan Regulation, please leave them in the box below.

52. **We may want to follow up** on some of the survey responses to elaborate further. **Please provide your contact details** below if we may contact you regarding this study.

**Name** 

**Email Address**

**Thank you for completing the survey!**

Bas du formulaire

Bas du formulaire

Bas du formulaire