IRDiRC Task Force on Sustainable Economic Models in Drug Repurposing

This survey will be used to elicit the specificity of Sustainable Economic Models in Drug Repurposing. The scope includes understanding the key features of drug repurposing projects and the corresponding business and/or funding models (depending on the available cases, current or past repurposing approaches), and the subsequent presentation of recommendations on how to adjust/optimize these models so to ensure a sustainable approach to drug repurposing.
The survey includes 73 questions.
Your personal information and data are collected by Inserm which is responsible for the Scientific Secretariat of IRDiRC. Your personal information and data are collected according to the European General Data Protection Regulation rules.
If you require further details, please contact scisec-irdirc@ejprarediseases.org (mailto:scisec-irdirc@ejprarediseases.org)

* Required

Identification of the person completing the survey

1. First Name and Last Name *

2. Email address *

3. Affiliation (e.g. Institution Name) *

Description of the Company/Organization

4. What is the focus of your company/organization? *

5. Is your company/organization fully or partially dedicated to repurposing activities? *

- ◯ Fully
- ◯ Partially

Description of the drug repurposing approach and strategy

6. What was the trigger of your repurposing approach? (check all that apply) *

- [ ] Extensive literature evidence
- [ ] Exciting scientific discovery
- [ ] Datamining
- [ ] Existing off-label use
- [ ] Clinical observation
- [ ] Extend the protection of a patent owned by your company/organization
- [ ] Patient need
- [ ] Other

7. If other triggers stimulated the repurposing approach, please describe them below.

8. Have you used or considered one of the following repurposing approaches? (check all that apply) *

- [ ] Computational
- [ ] Omics-based
- [ ] Network-based approach
- [ ] Data-driven
- [ ] Hypothesis-driven
- [ ] Other

9. Please comment the choice of your repurposing approaches. *

10. What was the repurposing strategy of your product relating to? *

- ◯ A classic case of 2nd medical use where the drug was initially developed and approved in US/EU for another indication
- ◯ A drug that was previously developed for a specific indication but did not demonstrate efficacy and was thus abandoned (clinical trial studies were already advanced with completion of phases I and/or II) but could be suitable for a new indication
- ◯ A drug developed for a specific indication having encountered safety issues and was abandoned but could be suitable for a new indication
- ◯ A previous development was abandoned because of financial issues or other commercial considerations
- ◯ Other

11. If another repurposing strategy was used, please describe it below.

12. Was the future availability of the drug for patients (once the drug repurposing is completed) an aspect that you took into consideration at the start the project? *

- ◯ Yes
- ◯ No

13. If no, please describe why.

Identification of the stakeholders

14. Who were the different stakeholders involved in the drug repurposing project? *

15. In which phases of the repurposing project were the different stakeholders involved? *

16. How were patient organizations involved in this new treatment development? (check all that apply) *

- [ ] Initiated research through fundraising
- [ ] Approached by the company to provide input in the protocol development
- [ ] Approached by the company/clinical site to support patient recruitment in the study
- [ ] Other

17. If the patient organization(s) was/were involved by any other way, please describe it below.

18. What impact did patient involvement have on the timeline of the treatment development? *

- ◯ No impact
- ◯ Some impact
- ◯ Significant impact

19. Please comment and/or share examples. *

20. What impact did patient involvement have in the development and the uptake of the product in the market? *

- ◯ No impact
- ◯ Some impact
- ◯ Important impact

21. Please comment and/or share examples. *

22. In hindsight, do you think patient organization(s) should have played a bigger role in the treatment development? *

- ◯ Yes
- ◯ No

23. What could have been done better to engage patients in this drug repurposing?

Required evidence generation and drug repurposing research studies

24. Did you have to generate additional evidence for the conduct of the repurposing research studies? (check all that apply) *

- [ ] Toxicology testing
- [ ] Pre-clinical pharmacology testing
- [ ] In vivo pre-clinical proof of concept
- [ ] Other

25. Please describe why these additional evidence had to be generated (e.g. higher dosing, lack of in vivo mechanisms of action). *

26. Did an animal model need to be developed? *

- ◯ Yes
- ◯ No

27. If an animal model was developed, please describe why.

28. Was the development of a new formulation required prior to initiation of clinical trials? *

- ◯ Yes
- ◯ No

29. If a new formulation was required, please describe why.

30. Which evidence were required for the regulatory approval of the clinical trials? *

31. Did you have to conduct a Phase 1 study in healthy volunteers? *

- ◯ Yes
- ◯ No

32. Were you able to run an abbreviated program, i.e., jump right to confirmatory study in patients with the condition? *

- ◯ Yes
- ◯ No

33. What type of endpoints did you use? *

34. How many efficacy trials were required to support the new indication? *

35. How long did it take to go from first-in-indication testing to marketing approval? *

Please describe in brackets if the first-in-indication testing was at the pre-clinical or clinical stage.

Characterization of the drug repurposing sources and mechanisms of funding

36. Which sources of funding did you use for the drug repurposing project? (check all that apply) *

	No Contribution	Low Contribution	Medium Contribution	High Contribution
The company/organization own funding	◯	◯	◯	◯
Public funding (e.g. Government)	◯	◯	◯	◯
Capital risk companies	◯	◯	◯	◯
Patient Associations	◯	◯	◯	◯
Academic Funding	◯	◯	◯	◯
Non-for-Profit	◯	◯	◯	◯
Other	◯	◯	◯	◯

37. If other funding sources were used, please identify them below (describe in brackets their level of contribution).

38. Which mechanisms of funding did you use for the drug repurposing project as a whole? (check all that apply) *

A cooperative agreement is an award similar to a grant, but in which the sponsor anticipates having substantial involvement in research activities once the award has been made.
A public-private partnership is defined as a collaboration between a public institution and a private-sector company that can be used to finance, build, and operate projects.
Equity funding is defined as the process of raising capital through the sale of shares.

	No Contribution	Low Contribution	Medium Contribution	High Contribution
Grants	◯	◯	◯	◯
Cooperative Agreement	◯	◯	◯	◯
Contract Agreement with Industry	◯	◯	◯	◯
Public-Private Partnership	◯	◯	◯	◯
Loans	◯	◯	◯	◯
Philantropy/Donation	◯	◯	◯	◯
Charity Fundraising	◯	◯	◯	◯
Company/Organization Fundraising	◯	◯	◯	◯
Equity Funding	◯	◯	◯	◯
Own funds or resources	◯	◯	◯	◯
Other	◯	◯	◯	◯

39. If other funding mechanisms were used, please identify them below (describe in brackets their level of contribution).

40. Amongst those, which mechanism(s) of funding did you use for the planning and conducting the clinical trials? (check all that apply) *

- [ ] Grants
- [ ] Cooperative Agreement
- [ ] Contract Agreement with Industry
- [ ] Public-Private Partnership
- [ ] Loans
- [ ] Philantropy/Donation
- [ ] Charity Fundraising
- [ ] Company/Organization Fundraising
- [ ] Equity Fundraising
- [ ] Own funds or resources
- [ ] Other

41. If other funding mechanisms were used for the planning and conducting the clinical trials, please identify them below (describe in brackets their level of contribution).

Sustainability of the economic model chosen

42. Would you say that the economic model you chose for the repurposing approach offers good sustainability? *

- ◯ Yes
- ◯ No

43. If affirmative, please indicate who can benefit from this sustainable economic model? (check all that apply)

- ☐ Company/Organization
- ☐ Payers/Health Care System
- ☐ Patients

44. If affirmative, please indicate by which means this sustainable economic model offers benefits to the above mentioned stakeholders.

Patent status and exclusivity periods

45. What was the patent status of the drug? *

- ◯ Not granted (still under procedure of granting the patent)
- ◯ Granted
- ◯ Expired/Abandoned
- ◯ Do not know

46. If a patent was granted for the drug, how many years of protection the patent had left?

- ◯ Under 5 years
- ◯ 5-10 years
- ◯ 10-15 years
- ◯ 15-20 years
- ◯ Do not know

47. Did the patent status of the drug interplay in your strategic approach? *

- ◯ Yes
- ◯ No

48. If affirmative, please describe how it interplayed in your strategic approach.

49. At which stage of your repurposing research programme have you taken into account the industrial protection of the drug? *

- ◯ Early on while choosing the repurposing approach
- ◯ After screening of potential candidates
- ◯ After the first preliminary data that resulted into positive outcomes
- ◯ Other

50. If the industrial protection of the drug was taken into consideration at another stage, please describe it below.

51. At which stage did you seek a freedom-to-operate analysis to make sure you could commercialize on this research? *

- ◯ Early on while choosing the repurposing approach
- ◯ After screening of potential candidates
- ◯ After the first preliminary data that resulted into positive outcomes
- ◯ Other

52. If the freedom-to-operate study was requested at another stage, please describe it below.

53. If you had to fill a patent, which protection strategy did you choose?

- ☐ Product with specific/new medical use
- ☐ Product in combination with another drug
- ☐ New formulation or prodrug, medical device
- ☐ Combination with a medical device
- ☐ Production method of the product
- ☐ Specific dosing
- ☐ New route of administration
- ☐ Other

54. Please describe why did you choose this strategy for patenting the drug?

55. Do you have a predetermined strategy for the life of the patent?

- [ ] Exploiting it and make good use of its monopoly (e.g. manufacture it and distribute it)
- [ ] Licensing it
- [ ] Selling it
- [ ] Other

56. Please describe why did you predetermine this strategy for the patent life?

57. Did the repurposed drug benefit from a period of orphan drug market exclusivity? (Check all that apply) *

- [ ] Yes - USA
- [ ] Yes - EU
- [ ] No

Identification of the barriers and challenges

58. What are the major obstacles you haven encountered in the drug repurposing project? *

- [ ] Collection of scientific evidence to support the repurposing approach
- [ ] Lack of data sharing
- [ ] Availability of the product/drug for the study
- [ ] Identification and engagement of stakeholders
- [ ] Selection and implementation of the economic model
- [ ] Conduct of pre-clinical research studies
- [ ] Conduct of clinical trials
- [ ] Regulatory aspects
- [ ] Licensing of the new therapeutic indication by regulatory agencies
- [ ] Change of the patent status
- [ ] Other

59. If other barriers have been encountered during the repurposing process, please describe them below.

Measure of progress

60. Did you use key performance indicators (KPI) for each of the repurposing phase? *

- ◯ Yes
- ◯ No

61. If affirmative, please describe the KPI below.

62. Did you introduce milestones during the repurposing process? *

- ◯ Yes
- ◯ No

63. If affirmative, please describe the milestones below.

Results of the drug repurposing process

64. What was your success outcome? *

65. Were you able to repurpose the drug to a new indication that reached patients? *

- ◯ Yes
- ◯ No

66. Were you able to extrapolate to other additional indications? *

- ◯ Yes
- ◯ No

67. Please describe whether it was associated with regulatory approval and/or mentioned in consensus guidelines. *

68. What is the commercialisation status / availability of the drug nowadays in terms of drug licence and reimbursement (e.g. available to patients in several countries)? *

69. Did the repurposing of the drug affect the pricing? *

Recommendations for a successful drug repurposing process

70. Could you describe which steps of the drug repurposing advanced very positively and why the whole repurposing process was successful? *

71. Would you have any tips or retrospective analysis that you would like to share with us if you were to adjust or optimize the choices you have made? *

72. What would in your view, given your expertise in this field, make a Sustainable Economic Model for Drug Repurposing? *

73. Do you have any other recommendations?