

**Participant Information Sheet**

**Title of study:**

Stakeholders’ perspectives toward obstacles and opportunities associated with accessing Orphan Drugs in Northern Ireland - A Qualitative Study

**1. Background**

Rare diseases affect approximately 6% of the worldwide population, which is >110,000 people across Northern Ireland (NI) at some point in their lives. An ‘orphan drug’ is the name given to a medicine developed to help prevent or treat a rare disease. Orphan drugs are available for <5% of rare diseases, but peoples’ report challenges accessing appropriate treatment options & pharmaceutical companies anecdotally describe issues bringing clinical trials for orphan drugs to NI.

You are invited to be a part in this project because you are living in Northern Ireland and have knowledge about obtaining orphan drugs for persons living with a rare disease either because of your work or your experiences as an individual with rare disease or caregiver.

This leaflet provides important information you will need to think about when deciding whether to take part in this project. Please read it carefully as you consider whether to join this study. You should not agree to take part in this research until you have had all your questions answered satisfactorily. If you require further information or would like this information in another format, please contact us as below.

Ghada Abozaid. raredisease@qub.ac.uk Principal Researcher, Centre for Public Health, QUB

Amy Jayne McKnight raredisease@qub.ac.uk Chief Investigator, Centre for Public Health, QUB

**2. What is the purpose of the study?**

The purpose of this study is to identify barriers and enablers related to accessing orphan drugs, and to help develop ways to improve orphan drug availability in NI.

**3. Why should I participate?**

You should consider participating if you have relevant experience about orphan drugs for rare diseases. Your knowledge and experience can contribute greatly to identifying obstacles to accessing orphan drugs in NI and help identify how we may overcome them. We also welcome hearing success stories from people who live in NI and have accessed orphan drugs.

**4. Do I have to take part?**

*No. It is up to you to decide whether or not to take part. If you do decide to take part, you will be given this information sheet to keep and you will be asked to sign a consent form or provide recorded verbal agreement before commencing a telephone, video, or face-to-face interview. If you choose to take part, you can change your mind at any time and withdraw from the study without giving a reason. A decision to withdraw will not affect the standard of care you receive.*

**5. What will happen to me if I take part?**

If you decide to participate in this study, a researcher will contact you to discuss the details of the study, the interview process, schedule the interview at a time that is convenient for you and answer your questions. The interview will take place either in person at a location of your choice (including a private room in the Centre for Public Health, Queen’s University Belfast, located near the Royal Victoria Hospital); or by telephone or online via Teams or Zoom. If you decide to have face-to- face interview, the interviewer will contact you the day before the interview to check if you have any respiratory infections including COVID-19 symptoms such as a continuous cough; high temperature, fever, or chills; loss of or change in, your normal sense of taste or smell; shortness of breath; unexplained tiredness, lack of energy; muscle aches or pains that are not due to exercise; not wanting to eat or not feeling hungry; headache that is unusual or longer lasting than usual; sore throat, stuffy or runny nose; diarrhoea; or feeling sick or being sick. If you have any of these symptoms the interview process will be postponed or virtual based on your status and agreement. No in person interviews will take place if the interviewer has any signs or symptoms of being unwell.

The interview will last approximately 60 minutes and will be guided by a list of questions from the researchers; this is known as a semi-structured interview, which is designed to be flexible. If you do not wish to answer any of the questions during the interview, you may say so and the researcher will move on to the next question. You may also ask that the interview stop, and either resume shortly, at another time, or not at all. You also have the right to withdraw your data from the study at any time without giving a reason; if the data has already been analysed in an anonymised format, then we can no longer identify individual participants to withdraw their data.

The questions will be straightforward and simple, asking about opportunities and problems in accessing orphan drugs for treating rare diseases and how to overcome them. You will then be asked to answer questions about your background so we can describe the range of people we interviewed while ensuring their identities will remain unknown (anonymous). The interviews will be digitally recorded after your consent, but all identifying information will be removed from the data prior to analysis. Only anonymised data will be used for evaluation and reporting of results.

Travel expenses and refreshments will be covered for those attending an in-person interview. Participants undergoing the interview will be eligible to receive voucher for £100 for their time and expenses undergoing the interview.

**6. What are the possible risks or disadvantages of taking part?**

There are no expected risks of participating in the study. You will be spending 60 minutes of your time being interviewed. COVID- 19 prevention protocol will be taken in our consideration by keeping the social distance at least 2 meters, interviewer will wear a mask, sanitizers readily available, and wiping down the shared surfaces (pen, desk, and chair) between interviewees.

In addition, some of the interview questions might cause psychological distress, anxiety or might be challenging, but this is not expected. If that is the case, you can choose to not answer those questions and the researcher will move to the next one. You can also ask the researcher to stop the interview and resume later or not at all.

**7. What are the possible benefits of taking part?**

There is no intended direct benefit to participants from taking part in this study. However, the information you provide may help to improve the orphan drug accessibility for persons living with a rare disease. This improvement may result in better quality and length of life for people with rare diseases that have an orphan drug available or where a medicine may become available in the future.

**8. What if something goes wrong?**

**We do not anticipate anything going wrong that will harm participants, however we appreciate incidents may occur at any stage.** If this study has harmed you in any way, or if you wish to make a complaint about the conduct of the study you can contact the Chief Investigator at Queen’s University Belfast using the details below for further advice and information:

Prof Amy Jayne McKnight, Telephone: 028 9097 6359, Email: [a.j.mcknight@qub.ac.uk](mailto:a.j.mcknight@qub.ac.uk)

Should you remain unhappy and wish to make a formal complaint, you can contact the Research Governance Team at Queen’s University Belfast (Tel: 028 9097 2529; Email: [researchgovernance@qub.ac.uk](mailto:researchgovernance@qub.ac.uk)).

**9. Will my taking part in this study be kept confidential?**

The audio files from the recorded interviews will be kept in password-locked and encrypted computer files. Once they have been used to create a word-for-word (verbatim) written document, they will be erased in order to protect participants’ identities. Printed copies of study material will be kept in a locked filing cabinet in the Molecular Epidemiology and Public Health Laboratories at the Institute of Clinical Sciences Building A, until the study is finished.

All study information is confidential, and no one except the study team will have access to it. If you change your mind and decide that you no longer want to take part in the study, you may withdraw your data from the study at any time. You will not have to give a reason for your withdrawal.

**10. What will happen to the results of the research?**

The results and findings from this research may be included as part of the researcher’s PhD theses, disseminated through scientific publications, conferences and seminars. If you like, a copy of the study results can be sent to you once the study is completed.

**11. Who is organising and funding the research?**

This project is part of a PhD thesis which is sponsored by Queen’s University Belfast.

**12. Who has reviewed the study?**

This study has been reviewed by two expert peer reviewers and the Faculty of Medicine, Health & Life Sciences (MHLS) Research Ethics Committee.

**13. Contact for Further Information**

If you have any questions or require more information about this research, please contact me or my research supervisors using the following contact details

Principal Project Investigator contact: Ghada Abozaid, [raredisease@qub.ac.uk](mailto:gabozaid01@qub.ac.uk); +44 (0)2890 976359

Queen’s University Belfast Rare Disease team contact: [raredisease@qub.ac.uk](mailto:raredisease@qub.ac.uk)

*This research will be conducted in compliance with data protection legislation. For more information about how we look after your information, how to access your rights and who to contact if you have any queries or concerns about data protection please visit the Queen’s University Belfast website* - [www.qub.ac.uk/privacynotice/Research/ListofResearchPrivacyNotices/PrivacyNoticeforResearchParticipants](http://www.qub.ac.uk/privacynotice/Research/ListofResearchPrivacyNotices/PrivacyNoticeforResearchParticipants)

**Thank you for your interest in this study and for taking the time to read through this information sheet.**