# DATA REQUEST FORM

In order to submit a request for access to patient-level data, please complete this form in English and do not use abbreviations. If you already have a protocol or analysis plan for the research, please "cut" and "paste" plain text from that document to complete this form.

Required fields are marked with an (*)

A. * Have you previously submitted or plan to submit an enquiry to add studies to this research proposal?

○ Yes ○ No

B. * Have you received an answer to your enquiry?

○ Yes ○ No

*Please provide the enquiry Reference Name and Number:*

C. * Is this research proposal a re-submission of a previous research proposal that has been reviewed by the Alexion Review Team?

○ Yes ◉ No

*Please provide the research proposal reference name and number:*

n/a

D. * Does your proposed research require Ethics Committee or Institutional Review Board (IRB) approval? (*If No, please provide further details below*).

○ Yes ○ No

☐ * I understand that the data are primarily provided in the secure data access system. SAS, "R" and "Plink" statistical software are provided, and there are controls to prevent the original study datasets from being downloaded.

☐ * I acknowledge that I, and the legal staff of my choice and of any relevant institution, have reviewed and understood the terms of use that apply in the data sharing agreement. I also understand that if I, or any member of my research team, fail to comply with the terms of the data sharing agreement, Alexion reserves all rights identified in the data sharing agreement including, but not limited to sharing information related to the aforementioned failure to comply with individual(s) or company(s) of Alexion's choosing including, but not limited to, other pharmaceutical and medical device companies.

☐ * By submitting this research proposal, I accept that the name and affiliation of the lead researcher, the title of the proposed research, the requested studies, research proposal summary, funding source and any potential conflicts of interest that are provided may be published on one or more sites of Alexion's choosing. For approved requests, the statistical analysis plan for the proposed research, and the publication citation may be posted on one or more sites of Alexion's choosing after the research is published.

## 1) RESEARCH PROPOSAL TITLE

* Please provide a full research proposal title. This should be descriptive and understandable to the general public and should reflect the aim of the proposed research.

*(500 character limit. Please create and attach Appendix A to input overflow.)*

## 2) RESEARCH PROPOSAL SUMMARY IN PLAIN ENGLISH

* Please provide a brief, high-level, overview of the planned research written in plain english language. This should be suitable for the general public and may be posted on one or more sites of Alexion's choosing after the Data Sharing Agreement has been signed.

Please include:

- Succinct details of the objectives of the proposed research including any hypotheses being tested.
- A clear summary of the background to this research including a discussion of the questions/issues or knowledge gaps and, if applicable, any unmet medical need(s) or public health issues to be discussed, how many patients / members of the public are potentially affected, how will the research add to medical science or patient care.

*(500 character limit. Please create and attach Appendix B to input overflow.)*

## 3) RESEARCH PLAN PROPOSAL

### a) * Research Background

Please provide a summary of the research proposal background. This section should include how this research will contribute to an unmet need or fill a gap in medical knowledge. Please provide references to prior work (if applicable).

*(500 character limit. Please create and attach Appendix C to input overflow.)*

b) * Research Objectives

Please provide details of the main objectives and the outcomes being evaluated including any hypotheses being tested. If your research proposal is not assessing specific outcomes, options may include:

- New research question to examine treatment effectiveness on secondary endpoints and/or within subgroup populations
- New research question to examine treatment safety
- Research that confirms or validates previously conducted research on treatment effectiveness
- Research that confirms or validates previously conducted research on treatment safety
- Preliminary research to be used as part of a grant proposal
- Summary-level data meta-analysis
- Participant-level data meta-analysis

Please specify if you only need summary-level data or specific documents (or category of documents) related to the study. *(500 character limit. Please create and attach Appendix D to input overflow.)*

c) * Study Design

Please provide a brief description of the study design, for example:

- case-control
- cohort
- cross-sectional
- historical controlled
- hybrid designs
- meta-analysis
- pooled analysis

Please also provide a description of the study population or populations for the proposed research, for example:

- the study arms from the requested clinical studies;
- intent-to-treat or per-protocol populations;
- the inclusion and exclusion criteria for any cohort or subgroup analysis.

Please clarify how the data you have requested will help you answer the hypothesis you have proposed. This includes outlining the methods you will use and how you will handle any missing data. Consider providing well defined baselines and SAP details that consider as many variables as possible.

d) * Do you plan to include non-Alexion data (i.e., data not obtained wholly or partial from Alexion in this research proposal) in your research project?

○ Yes ○ No

i) If yes, please provide a list of non-Alexion study data and sources.

Please provide a list of any non-Alexion study data that you wish to include in this research proposal. Also provide the source of these data, the associated sample sizes, and the interventions of interest that were studied.

Please include as much detail as possible to identify the non-Alexion study data including sponsors, titles , trial identification numbers, and other study identifiers (*such as NCT numbers for each study and/or if needed PubMed IDs/references*).

*(500 character limit. Please create and attach Appendix E to input overflow.)*

ii) * Data Management Plan for combining non-Alexion data with Alexion data

Please provide a plan for how you intend to combine or otherwise compare these external study fields with the study data provided by Alexion.

*(500 character limit. Please create and attach Appendix F to input overflow.)*

e) * Rationale for Study Selection and Selection of Populations/Participants

Please provide your rationale for choosing each study (*Alexion and non-Alexion if applicable*).

This section should include the criteria for considering studies and specific populations for inclusion/exclusion in the proposal and may also include the following:

- Types of studies included in your analysis (phase, interventional vs non-interventional, controls etc).
- The interventions and comparisons of interest and the associated outcomes for each study.
- The study/population sizes for each study and any impact that may have on your analysis.
- Whether you intend to focus on intent-to-treat or per-protocol populations.
- Any relevant studies that were excluded and why.

*(500 character limit. Please create and attach Appendix G to input overflow.)*

## f) Statistical Analysis Plan

* Please provide a detailed description of the statistical methods that will be used.

This methodology description should not be limited to a list of tests. It should be a discussion of such items as:

- Effect measure of interest (e.g. for inferential studies: odds ratio, risk or rate ratio, risk or rate difference, absolute difference)
- Statistical analysis methods (e.g. logistic regression, Kaplan-Meier curves, log-rank test, multiplicity adjustments)
- Planned adjustment for covariates
- Meta-analysis methods, if applicable (e.g. random effects meta-analysis, stratified meta-analysis, meta-regression)
- Power to detect a clinically important effect, or the precision of the effect estimate given the sample size available
- Planned sensitivity analyses, if relevant
- Planned subgroup analyses [e.g. by age, disease status, ethnicity, socio-economic status, presence or absence of co-morbidities, different types of intervention (e.g. drug dose)]
- Handling of missing data

*(500 character limit. Please create and attach Appendix H to input overflow.)*

g) Publication Plan

* All research proposals must have a publication plan for the communication of the results. Please provide your plans for publishing and/or describe whether and how the outcome of your research will be communicated to the public.

*(500 character limit. Please create and attach Appendix I to input overflow.)*

## 4. RESEARCH TEAM DETAILS

Please note that a suitably qualified researcher with expertise in the statistical analyses being proposed must be a member of the research team. In some cases, this may be the lead researcher while in others a designated member of the team will be the appropriately qualified researcher. The qualified researcher usually has a degree in statistics or a related discipline (e.g. mathematics, health economics or epidemiology) but other qualifications or experiences may be considered relevant by the Alexion Revew Team.

For this section, please include all researchers on your team. You must notify us when there is a change in membership of the research team's lead researcher or qualified researcher after your proposal is submitted or approved.

Fees and licenses for research team members to access data in the secure data access system and use of the statistical software are paid by Alexion. The number of licenses for each research proposal is generally limited to five researchers per project. If more research team members are required, please consult with Alexion.

a) Lead Researcher

* Name:

* Email:

ORCID ID:

*The ORCID ID is a persistent digital identifier that distinguipshes you from every other researcher, and, through integration in key research workflows such as manuscript and grant submission, supports automated linkages between you and your professional activities ensuring that your work is recognized.*

*Post or Position:

*Employer/ Company/ Research Institution/ Relevant Affiliation:

* Education, Professional Qualifications, and memberships that are relevant to the proposed research:

*Potential conflicts of interest i.e. Financial relationships that may or may be perceived to influence the planning, conduct or interpretation-bias of the proposed research. This might include board memberships, consultancies, employments (e.g. working for competitors), grants/ grants pending, patents, royalties etc.)*

Please summarize how real or potential conflicts of interests or biases will be managed:

b) Lead Statistician or Qualified Researcher

* Name:

* Email:

* Post or Position:

* Employer/ Company/ Research Institution/ Relevant Affiliation:

* Education, Professional Qualifications, and memberships that are relevant to the proposed research:

*Potential conflicts of interest i.e. Financial relationships that may or may be perceived to influence the planning, conduct or interpretation-bias of the proposed research. This might include board memberships, consultancies, employments (e.g. working for competitors), grants/ grants pending, patents, royalties etc.)*

Please summarize how real or potential conflicts of interests or biases will be managed:

c) Additional Researcher(s)

* ◯ Yes ◯ No

*Note: Please create and attach Appendix K to include additional information about the Researcher(s).*

d) Source(s) of Funding for the proposed research:

* Please provide the funding source(s) that are being used or are planned to be used solely or in part for the proposed research. Please record all funding sources if there are more than one.

Please include research grants from governments or government agencies, other grants or

donations, funding from employers through employment contracts, other contracts, consultancies, honoraria and other payments that will be used for the research .

Please include any funding from commercial (e.g. for profit) organizations. If there is no funding required for the research, enter "None".

*(500 character limit. Please create and attach Appendix L to input overflow.)*

e) Other Relevant Information:

Please provide any additional information that should be considered when reviewing this proposal:

*(500 character limit. Please create and attach Appendix M to input overflow.)*

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