

## EMD Serono Position Statement on Responsible Clinical Trial Data Sharing *For External Use*

### Why it Matters

EMD Serono believes that as a biopharmaceutical company, the sharing of information related to company sponsored Clinical Trials is central to our mission. The sharing of clinical trial information enables the medical and scientific community to further develop the medical and scientific knowledge base and permits the public to make informed healthcare decisions. It is also one of the best ways to inform prescribers and patients about scientific findings relating to our medicines.

We are committed to enhancing public health through responsible sharing of clinical trial data in a manner that is consistent with: (a) safeguarding the privacy of patients (b) respecting the integrity of national regulatory systems and (c) maintaining incentives for investment in biomedical research.

### EMD Serono Position Statement

- EMD Serono sponsored trials are those studies in which our company is ultimately responsible for all aspects of the study, even if some or all of these activities are transferred to another party (such as a contract research organization).
- **Clinical Trial Registration** –
  - **What:** EMD Serono registers the designs of all of its clinical trials in patients<sup>1</sup>.
  - **Where:** EMD Serono registers this information on the publicly accessible website [www.clinicaltrials.gov](http://www.clinicaltrials.gov), maintained by the U.S. National Institute of Health (NIH), and other applicable websites as required. This allows healthcare providers, patients and their families to easily locate clinical trials.
  - **How:** EMD Serono commits to assign each trial a unique identifier (e.g., a company-assigned study ID) to ensure transparency and allow users to track the trial through multiple databases, including clinical trial results databases.
  - **When:** These clinical trials are registered before the trial starts.
- **Clinical Trial Results Disclosure** –
  - **What:** EMD Serono discloses the results of all clinical trials in patients once a product has been approved for marketing and is commercially available in at least one country, regardless of outcome. Investigational products whose development programs are discontinued, will also be disclosed, regardless of outcome.

<sup>1</sup> The most important clinical trials are those that test a medicine on subjects who actually require medical care: patients. The results of trials such as these are integral to drug development, because they provide medical evidence regarding the safety and effectiveness of medicines in the population intended to use the medicine. These are the clinical trials for which EMD Serono commits to providing registry and results information.

- **Where:** EMD Serono makes this information available on the publicly accessible website [www.clinicaltrials.gov](http://www.clinicaltrials.gov), maintained by the U.S. National Institute of Health (NIH), and EudraCT, maintained by the European Medicines Agency and other applicable websites as required.
- **How:** In all cases, disclosures will be undertaken in a manner consistent with applicable national laws and rules governing protection of patient privacy and intellectual property.
- **When:** EMD Serono discloses the results to [clinicaltrials.gov](http://clinicaltrials.gov) within 30 days after approval of the drug, or within 12 months after a trial ends, whichever occurs first. For trials with marketed products that are terminated prior to completion, information will be provided on the date of cessation and reasons leading to the decision to discontinue the trial.
- **Clinical Study Reports** - Following approval of a product and indication in both the European Union and the United States, EMD Serono will make publicly available Clinical Study Report synopses that were filed with regulators on or after January 1, 2014.
- **Clinical Trial Participant Communication** - EMD Serono will support investigators in providing their research participants with a summary of the trial results after conclusion of the trial. In addition, EMD Serono will work with regulators to adopt mechanisms for providing a factual summary of clinical trial results and make the summaries available to study participants.
- **Investigator Access to Data and Review of Results** - EMD Serono values the significant contribution of each clinical investigator without whom advancements in medical science would not be possible. We seek to provide our investigators access to clinical data from the studies in which they participate.
  - Individual investigators in multi-site clinical trials will have their **own research participants' data**, and will be provided the randomization code after conclusion of the trial.
  - We will provide a **summary of the study results** to the investigators.
  - Any investigator who participated in the conduct of a multi-site clinical trial will be able to **review data for the entire study** at the sponsor's facilities, or other mutually agreeable location in response to a reasonable scientific inquiry.
  - Investigators who are authors of study-related manuscripts will be given **all study data needed to support the publication**.
- **Information Shared with Researchers** – Following approval of a new product or a new indication for an approved product in both the European Union and the United States after 1 January 2014, EMD Serono will share study protocols, anonymized patient level, and study level data and redacted clinical study reports from clinical trials in patients with qualified

scientific and medical researchers, upon researcher request, as necessary for conducting legitimate research. Data will not be shared for products and indications approved prior to the effective date of this Policy. In addition, data will not be shared with EMD Serono competitors.

- **Publications** – All EMD Serono clinical trials in patients will be considered for publication in the scientific literature, regardless of outcome. In addition, this commitment also pertains to investigational medicines whose development programs have been discontinued.

## Frequently Asked Questions:

1. **Question:** What Platform is EMD Serono using to share its clinical trial data?

**Answer:** EMD Serono is registering the design of all clinical trials in patients as well as the summary results for these trials on the website [www.ClinicalTrials.gov](http://www.ClinicalTrials.gov) maintained by the U.S. National Institute of Health (NIH), and other applicable websites as required. Trial results for an investigational product that has failed in development will also be posted to this website.

In addition, EMD Serono has agreed to share anonymized patient level clinical study data, study level clinical data and protocols, to qualified researchers for the purpose of advancing science and most importantly, benefiting patients. Currently, we are sharing this data via our webportal.

2. **Question:** Why hasn't EMD Serono joined the platform [Clinicalstudydatarequest.com](http://Clinicalstudydatarequest.com) like Bayer, Boehringer Ingelheim, GSK, Novartis, Roche, Sanofi and ViiV Healthcare?"

**Answer:** There are several industry/academic/consortia led platforms being developed for the purpose of Data Sharing. Like many pharma companies, EMD Serono is evaluating these options.

3. **Question:** Can you provide more details about the type of information EMD Serono is prepared to share with qualified medical and scientific researchers in response to a legitimate request?

**Answer:** EMD Serono is committed to sharing with qualified medical and scientific Researchers, patient-level data, study level data, and clinical study designs and protocols.

- Patient-level data refer to information on individual patients collected during a clinical study and recorded on case report forms (CRFs) and inputted into electronic databases or captured directly in electronic format, where it can be readily organized into patient-level listings and datasets (e.g., demography and baseline characteristics, exposure, medical history, concomitant medications, clinical

efficacy, safety events and safety lab, biomarker and pharmacogenetic). Data gathered depends on the study protocol (clinical study design information and protocol). This information is handled through what the Institute of Medicine (IOM) has described as a process by which data in a clinical study originate with CRFs, either handwritten or electronic, then go through several stages of auditing, queries, and refinement by original investigators and study staff to resolve ambiguities, and then ultimately yield “individual participant data.”<sup>2</sup>. This is typically available by sharing the anonymized raw and analysis datasets, together with the related metadata and the Statistical Analysis Plan.

- Study-level data consist of patient-level data that have been amalgamated, compiled and tabulated, transformed, stratified, or otherwise organized into study-level data sets, to be used in interpreting the outcome of a clinical study. Study-level data present clinical trial data in an objective manner, without subjective analysis or interpretation, usually in tabular, graphic, or statistical form showing, for example, averaged, stratified, or patterned presentations of study data gathered. Examples would include a table that presents cross-patient data on baseline patient characteristics (demographic and disease-related), patient disposition (i.e., numbers/percentages of patients who completed or discontinued the trial), endpoints (primary, secondary, and other), study drug exposure, adverse events, vital signs, and laboratory and other safety measures provided for the overall study population, and by subgroups. This is typically available through the final version of the study report.
- Clinical study design information and protocols direct investigators how to run a particular study. Protocols give instructions to the investigators on, for example, what drug to give and when, what study measurements to take and when and how to record them, and how to treat and record adverse events.

4. **Question:** What is the rationale for providing the synopsis of Clinical Study Reports (CSR)? Why not provide the full CSR as provided to the regulator(s)?

**Answer:** Given the volume of data contained in regulatory submissions – often containing millions of pages – EMD Serono commits to publishing a synopsis after marketing approval in the US, EU, or member states. The synopsis will provide patients and their physicians with enhanced information about the results of clinical trials and the evidence used to approve a new medicine. The synopsis is a part of the CSR and is reviewed by the FDA and EMA as part of their approval. In order to accelerate research and advance scientific understanding, EMD Serono will also evaluate requests for full CSRs, including patient-level and study-level data, and share them under the commitment outlined in “**Information Shared with Researchers**”.

<sup>2</sup> Institute of Medicine, Sharing Clinical Research Data: A Workshop Summary 10 (2013).

5. **Question:** Why may it be necessary to limit the availability of patient-level data for clinical trials conducted involving patients whose data are likely to be re-identified?

**Answer:** Protecting the privacy of patients who participate in clinical trials is a critical consideration for EMD Serono's. It may be possible even for "anonymized" patient-level data to be re-identified using modern data mining techniques<sup>3</sup>. For this reason, EMD Serono generally withholds patient level information from disclosure when there is a reasonable possibility that patient privacy could be jeopardized. The risk of "re-identification" is significantly higher when the number of patients is small, such as is typically the case for trials involving patients with rare diseases, which may include as few as 25 or fewer patients.

6. **Question:** How will EMD Serono determine who can receive patient level data or other proprietary information?

**Answer:** Research requests must be submitted in writing to the EMD Serono portal. Following receipt, researchers' requests will be evaluated initially by an internal committee at EMD Serono, which may decide to approve the request. If the EMD Serono committee denies the request, the request will be escalated to the EMD Serono Scientific Review Board for a second review (de novo). The Board shall include scientists and/or healthcare professionals who are not employees of EMD Serono. The Board shall be responsible for reviewing the request to determine whether the request meets the criteria for researcher qualifications and legitimacy of the research purpose, notwithstanding the denial by the EMD Serono committee.