Annex A Statistics on Requests for Access to Documents Held by the EMA (2012–2020)

Affiliation	Number of requests in %								
Not-for-profit organisation	0	0	1	0	0	2	1	3	3
EU Institutions	1	1	1	0	0	0	0	0	0
Regulator outside EU	2	1	0	0	0	0	0	0	0
EU NCA	2	2	1	0	0	0	0	0	0
Patients or Consumer (general public)	8	3	3	3	7	17	12	8	6
Healthcare professional	4	3	2	3	3	6	4	4	4
Academia/Research institute	9	12	8	8	8	8	8	9	9
Legal	14	23	16	11	11	8	9	8	7
Media	17	13	13	7	5	3	4	4	2
Pharmaceutical industry	32	35	47	60	55	44	47	52	55
Consultant	10	4		2	10	11	14	12	14
Other	0	1	7	4	0	0	0	0	0
Total number of requests	281	290	416	701	823	865	822	783	597
Year	2012	2013	2014	2015	2016	2017	2018	2019	2020

Data source: the EMA Annual Reports (EMA. Annual reports and work programmes. https://www.ema.europa.eu/en/about-us/annual-reports-work-programmes. Accessed 22 Jul 2021. The EMA annual reports do not break down the statistics into the types of documents requested from the EMA)

D. Kim, *Access to Non-Summary Clinical Trial Data for Research Purposes Under EU Law*, Munich Studies on Innovation and Competition 16,
https://doi.org/10.1007/978-3-030-86778-2

Annex B Glossary of Terms Related to the Design and Methodology of Randomised Clinical Trials

Arm	A group or subgroup of the trial participants in a clinical trial that receives a specific health intervention (treatment), or no intervention, according to the trial protocol
Case report form	A document containing all data gathered and recorded on individual study subjects according to the variables set out in a trial protocol
Clinical trial	An experimental study designed to evaluate the treatment effect of medical intervention in humans
Confirmatory trial/ 'hypothesis-testing trial'	An interventional study designed to test a predefined research hypothesis and usually conducted in order to generate evidence on the safety and (or) efficacy of an investigational product
Clinical study report	A document prepared according to the standardised structure and submitted to support an application for drug marketing authorisation that contains inter alia the trial protocol, statistical analysis plan, efficacy and safety analyses
Effect size	A quantitative characteristic that correlates with the outcome variable and statistically describes the study hypothesis
Endpoints/outcome measures	Variables that can indicate biologic and pathogenic processes, or pharmacologic responses to a therapeutic intervention
Exploratory (hypothesis-generating) trial	An interventional study that can generate a hypothesis regarding a treatment effect and set the direction for future research
Individual patient data	Data recorded for each individual study subject according to the trial protocol
Meta-analysis	A formal evaluation of the evidence from two or more trials that generated comparable evidence
Null hypothesis	

(continued)

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	An assumption of the absence of the association between the predictor (the indirect measurement of the treatment effect) and the outcome of interest in the target population
Outcome of interest	A measurable effect of a medicinal intervention on the clinical manifestations of a disease (e.g. relief of symptoms, improvement in the quality of life)
Treatment effect	An effect on the state of health that is attributed to an intervention
Power/trial power	Statistical significance of evidence; the probability that the null hypothesis will be rejected if it is not true; the probability that the difference between the study groups will be identified, where such difference, in fact, exists
Protocol/clinical trial protocol	A document setting out objectives, design, methodology, statistical considerations, organisation, and implementation of a clinical trial
Sample size	The number of study subjects enrolled in a trial that can be estimated inter alia based on the calculation of the statistical power of the trial
Source data	All original records of trial results, observations or other evidence gathered in a clinical trial allowing to assess the trial performance vis-à-vis its objectives