EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Inspections, Human Medicines, Pharmacovigilance and Committees Division

Cystic Fibrosis Workshop - Appendix 1. Table of recommendations

Group 1. Recommendations & Actions table. Common data elements that are needed by all stakeholders: Data validation

Topic	Recommendation	Action	Owner
Medication-related information.	Record CF related medication, Start/stop date (month), reason for discontinuing treatment. Record medication for co-morbidities; medication name and indication	Future: Aim registry linkage with hospital and community dispensing records.	Registry Holders
Medication-related complications	Registries suitable for ADR signal investigation / follow up not typically for detection	On request from regulator or MAH; Agreed protocol; time limited investigation period	Regulators Registry Holders MAH
Clinical trials, compassionate & off-label medications	Ask & record clinical trial participation; Record compassionate, off-label medications & indications		Registry Holders
CF exacerbations	Record number of exacerbations per year		Registry Holders, MAH Regulators
Severity of CF at commencement of a new treatment	Record CF severity based on most recent measurements of severity parameters: FEV1; BMI; infection status (no repeat measures)	Agreement on severity parameters included	Registry Holders
Hospitalisation	Record number of CF-related days of		Registry

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5520
Send a question via our website www.ema.europa.eu/contact

Topic	Recommendation	Action	Owner
	hospitalisation		Holders
Microbiology	Record first time isolation of pseudomonas, chronic colonisation with pseudomonas, MRSA colonisation	For other pathogens, investigate on request from regulator or MAH; Agreed protocol; time limited investigation period	Regulators Registry Holders MAH
Genotype	Genotype information recorded.		Registry Holders
Transplantation	Continue monitoring if feasible, otherwise transfer to transplant registry		Registry Holders
Patient Reported Outcomes (PROs)	Recommend inclusion in registries	Agree on PRO elements; Standardise across registries	Regulators Registry Holders MAH Payers
Pregnancy	Record if pregnancy occurred & if so, minimum outcomes information	Include pregnancy and outcomes on the registry database.	Registry Holders
Patient mobility between CF centres	Transfer historical information to new registry	Aim linkage between CF centres.	Registry Holders

Group 2. Recommendations & Actions table. Informed consents, governance, data protection, individual data vs aggregated data

Topic	Recommendation	Action	Owner
Informed consents	For new registry patients, review whether the consent is broad enough for possible future situations: 1) potential uses of registry data in line with the applicable legislation; 2) situations where data might be shared in summary or at patient level 3) providing an option for	Review of current national and ECFSPR consent considering necessary evolution	Registry holders (ECFS) Registry Task Force

Topic	Recommendation	Action	Owner
	patients to request that re-use of their data is limited to certain purposes.		
Informed consents	Develop a policy on situations where sharing of summary or pseudo-anonymised raw data is acceptable	Policy to provide a transparent guidance to potential requesters such as regulators and MAH.	Registry holders Regulators MAH
Data Protection	Assess the impact of the forthcoming GDPR regulation on circumstances where data sharing can be allowed		Registry holders, MAH Registry Task Force
Governance	Registry holders should establish a centralised process with SOPs for requesting data and outlining the types of data that can be provided and their format	To set up a centralised process with supporting SOPs for requesting data to support consistency of responses.	Registry holders Registry Task Force
Governance	Agree on a common approach to the collection, reporting and sharing of post-authorisation/ pharmacovigilance data.	Establish a Pharmacovigilance working group	Registry holders MAH Registry Task Force
Governance	Regulators to be aware of the data that can be collected by registries	Regulator – Registry holder communication	Registry holders Registry Task Force
	Registry holders to understand regulators' requests to MAH	Regulator – Registry holder communication	Registry holders Registry Task Force
Governance	Communicate to the public the benefits and potential uses of the data arising from patient registries	Communicate registry benefits and information on new studies	Registry holders MAH Registry Task Force
Data sharing	Direct communications should take place between the registry holder, the MAH and the regulator to clarify the details and feasibility of regulatory data requests	MAH and Registry holders agree on and apply standard contractual agreements	MAH and Registry holders

Topic	Recommendation	Action	Owner
Utility of registries for regulators and MAH	When potential ADRs are identified, registry holders should encourage physicians to use their national ADR reporting channels	Remind physicians to report ADRs through the channels and tools in place in each country.	Regulators
Utility of registries for regulators and MAH	For a pharmacovigilance study or specific signal follow up, registries could participate in targeted monitoring	Case-by-case approach	
Utility of registries for regulators and MAH	Studies based on registry data should be registered into the EU PAS register.	Registry holders to list registry-based studies in the EU PAS register	Registry holders
Timelines	Regulators and MAH must understand time schedule for data provision.	Registry holders to provide a schedule for data provision	Registry holders
Timelines	When data are required urgently, national registries may be approached directly given the lead-time to upload data to the ECFSPR	Regulators to approach national registries when data is required urgently	Regulators Registry holders
Funding	Funding for providing data and services is an opportunity for long-term support of the registry.	Case-by-case approach	MAH Registry holders

Group 3. Recommendations & Actions table. Common procedures and registry interoperability, quality assurance to support regulatory evaluation and data analysis

Topic	Recommendations	Actions	Owner
Processes for Data upload into registries and Audits	Explore options to minimise the number of (manual) steps and duplications of data entry	Map and review the current processes at national level to see if steps could be removed or simplified. In long term, this could facilitate the generation of encounter data and increase the use of registries in post authorisation studies.	National registry holders ECFSPR
Processes for Data upload into	Annual report on data quality based on appropriate indicators	Include a section in the ECFSPR Annual report covering the data quality of the national registries	ECFSPR Data Quality Group

Topic	Recommendations	Actions	Owner
registries and Audits		based on agreed indicators	
Processes for Data upload into registries and Audits	Audit in all countries, clear governance	Organise audits of the national and EU registries on regular basis in order to guarantee data quality in line with EU standards Develop EU accreditation system?	National registry holders ECFSPR
Data quality	Agree on EU standards for Data quality indicators and terminologies/coding	Propose EU standards for Data quality indicators and consult with decision makers and advisory bodies	ECFSPR Data Quality Group
		Harmonise registries reporting requirements / guidelines	Regulators
Data quality	Strengthen communication on the benefits of registries and importance of healthcare professionals involvement in data quality insurance	Provide direct feedback to clinicians or create dashboards for healthcare professionals to view the evolution of their patients and understand the benefit of their contribution	National registry holders
Data quality	Agreement on all stakeholders expectations including HTAs and payers	Clarify who does what, when, what data, and develop clear communication and timelines	All stakeholders
		Mend the broken triangle through early and more direct dialogue between the EMA and Registry Holders rather than via MAH, e.g. creation of specific fora	All stakeholders