Early Access Programmes Europe

Pharma iQ
a division of IQPC

Early Access Programmes: Compliance Handbook

Early Access Programmes: Regulation Handbook

Navigating the nuances and complexities of early access programmes has proven to be a resource draining task for pharma firms. The dense regulatory requirements of course exist to ensure that medicines are fit for purpose and efficacious enough to bring value to patients. However, the challenge of merely grasping the web of compliance considerations for varying countries can hinder the path and lead time of these programmes. For example in some regions it is illegal to promote the access to unapproved therapeutic goods, like Australia.

Shekar Natarajan, Director and Group Lead - International Regulatory Strategy, of Shire Pharmaceuticals plc noted that referring to the region's agency website is a logical first step, however reaching out to affiliates local to the area for further insight may also be required as the information provided on requirements may not be accessible online or may not be expressed in a language that can be deciphered by the firm.

Ahead of this year's Early Access Programmes Europe conference, Pharma IQ expands its regulatory handbook to develop the industry's awareness of the various compliance requirements that surround the implementation of these programmes.

Early Access Programmes: Regulation Handbook

This Pharma IQ handbook showcases the requirement highlights for Early Access Programmes in various regions.

Note: Please remember this is a guide only, and should not be taken as legal advice, links to authorities sources are provided where possible.

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Austria

Legislation:	Federal Medicines Act, 2009
Name of Programme	Compassionate Use.
Responsible Government Agency	Federal Ministry for Safety in the Health Care System.
Restrictions:	Patients are to be considered for inclusion in clinical trials first. One phase III trial should be in progress for the indications requested.
Point of Initiation:	Treating physician
Duration of Authorization:	One year, with the possibility of extension
Required Reporting:	Adverse events must be reported within 15 days, immediate notify premature termination , final safety report.
Drug Pricing	No direct reference in the law
Reimbursement:	Case by Case
Payer:	Regional Sickness funds

Australia

Legislation:	**Therapeutic Goods Act 1989, Therapeutic Goods Regulations 1990, Therapeutic Goods (Medical Devices) Regulations 2002.**
Name of Programme	Special Access Scheme
Responsible Agency	Therapeutic Goods Administration
Restrictions:	Possible conditions include: the maximum dose that can be supplied, the time in which the approval is valid, notification of the discontinuation of treatment within 6 weeks of termination., principles from National Health and Medical Research Council's National Statement on Ethical Conduct in Human Research 2007 need to be met, adverse consequences rest upon the doctor and patient.
Point of Initiation:	Qualified Practitioner
Duration of Authorization:	28 days
Required Reporting:	Adverse reactions by the doctor within 15 calendar days of the event. 6 monthly reports to TGA on the supply of the goods under the schemes.
Drug Pricing	No direct reference in the law
Reimbursement:	the Commonwealth does not subsidise the cost typically
Payer:	N/A

Belgium

Legislation:	Royal Decree of April 25, 2014
Name of Programme	Compassionate Use.
Responsible Government Agency	Federal Agency for Medicines and Healthcare Products (FAMHP)
Restrictions:	No approved drugs can be feasible alternatives, the products must be undergoing clinical trials or as the subject of an application.
Point of Initiation:	Treating physician
Duration of Authorization:	Feedback in 55 working days
Required Reporting:	Adverse effects and the risk-benefit ratio will be constantly evaluated by the agency.
Drug Pricing	Can be reimbursed if conditions are met - these conditions include obtaining a positive cohort decision.
Reimbursement:	Yes if conditions are met - including a positive cohort decision.
Payer:	Unmet Medical Need

Denmark

Legislation:	Section 29 (1) of the Danish Medicines Actderal Medicines Act, 2009
Name of Programme	Compassionate Use Permit
Responsible Government Agency	Danish Medicine Agency(DKMA) for Safety in the Health Care System.
Restrictions:	Compassionate Use Permit can either apply to a single patient or for use in a specified indication at a hospital ward or at a doctor's practice.
Point of Initiation:	Treating physician
Duration of Authorization:	Proposed by the physician but could apply for up to 5 years
Required Reporting:	Adverse events
Drug Pricing	Free Pricing
Reimbursement:	On an individual basis
Payer:	Hospital budget

Estonia

Legislation:	Not formally authorised by national law
Name of Programme	Compassionate Use Programmes
Responsible Government Agency	Ravimiamet agency
Restrictions:	Quantity of product imported may not exceed the amount for 12 months
Point of Initiation:	Physician through the pharmacy where the product will be dispensed. Wholesaler needs to apply for special authorisation for import.
Duration of Authorization:	30 days
Required Reporting:	Adverse events
Drug Pricing	N/A
Reimbursement:	N/A
Payer:	N/A

France

Legislation:	**Article 83 -Regulation no.276/2004/CE for the "cohort" ATU. Article 5- Directive 2001/83/CE for the "nominative" ATU**
Name of Programme	Temporary Use Authorization (ATU).
Responsible Government Agency	Agence nationale De sécurité du médicament et des produits de santé (ANSM)
Restrictions:	A drug which has market authorization overseas is viable for one of the ATU when enrolling for a marketing authorisation in France. Positive risk to benefit ratio must be established.
Point of Initiation:	Prescribing Physician
Duration of Authorization:	One year, with possible renewal
Required Reporting:	Periodic report with safety and efficacy data
Drug Pricing	Free pricing or company must declare and negotiate the price to CEPS (Economic Committee of Health Products prior to the programme's initation.
Reimbursement:	100% of price. Difference refunded to payer on registration
Payer:	Social Security system - Hospital pharmacies

Germany

Legislation:	**Section 21 (2) no.6 AMG (German Medicine Act) and article 83 of the European Regulation EG (no.726/2004)**
Name of Programme	Hardship Case Programme (AMHV)
Responsible Government Agency	German Federal Institute for Drugs and Medical Devices (BfArM)
Restrictions:	Only on cohort basis. The drug must have undergone clinical trials abroad or in Germany, or be in the late process of approval by EMA.
Point of Initiation:	Prescribing physician through the hospital pharmacist by fax
Duration of Authorization:	One year, with possible renewal
Required Reporting:	Adverse events and a final report on safety and efficacy.
Drug Pricing	Free pricing
Reimbursement:	Case by Case
Payer:	Negotiated on case by case

Ireland

Legislation:	**No provision in legislation for compassionate use programmes for specific groups of patients.**
Name of Programme	-
Responsible Government Agency	Health Products Regulatory Authority (HPRA)
Restrictions:	There are exceptions for individual patients to access medicines prior to authorisation via their practitioner's direct responsibility.
Point of Initiation:	-
Duration of Authorization:	-
Required Reporting:	-
Drug Pricing	-
Reimbursement:	-
Payer:	-

Italy

Legislation:	Law no. 326 of 24 November 2003
Name of Programme	Compassionate Use. Fondo AIFA 5%.
Responsible Government Agency	Agenzia Italiana del Farmaco (AIFA)
Restrictions:	Orphan drugs must have been approved by EMEA or in late stage of centralized approval process.
Point of Initiation:	Specialist Centre
Duration of Authorization:	6 months, with possible extension.
Required Reporting:	No specific rules
Drug Pricing	Remains unclear – would be a matter for the Scientific Technical Committee and the Prince and Reimbursement Committee of AIFA.
Reimbursement:	Compassionate Use- Drug is supplied free of charge. Fondo AIFA5% - 100% by Health Authorities or AIFA
Payer:	AIFA

The Netherlands

Legislation:	Article 83 of Regulation 726/2004/EC
Name of Programme	Compassionate Use programme in Cohort
Responsible Government Agency	Medicines Evaluation Board (MEB)
Restrictions:	Overview of previous clinical data must be provided, whether an opinion has been proposed by the Committee for Medicinal Products for Human Use.
Point of Initiation:	Physician
Duration of Authorization:	programme is valid for 1 year after approval and can be renewed.
Required Reporting:	Possible adverse events must be reported
Drug Pricing	N/A
Reimbursement:	N/A
Payer:	N/A

Slovenia

Legislation:	Article 83 of Regulation 726/2004/EC
Name of Programme	Compassionate Use
Responsible Government Agency	Agency of the Republic of Slovenia for Medicinal Products and Medical Devices (JAZMP).
Restrictions:	the product should be authorised in a third country coupled with a report on the quality analysis. It must be intended for a group of patients. A statement from the sponsor pledging to supply the product to the end of the scheme and for 1 more year after this to all patients.
Point of Initiation:	Sponsor/ practitioner
Duration of Authorization:	30 days after receiving a completed application
Required Reporting:	Efficacy and adverse events
Drug Pricing	An application must contain a statement that the manufacture is making the product available on the program free of charge.
Reimbursement:	No reimbursement
Payer:	Sponsor

Spain

Legislation:	Real Decreto 1015 of 19th June 2009
Name of Programme	Temporary Use Authorization / Named Patient Basis
Responsible Government Agency	Spanish Agency of Medicines and Health Products (AEMPS)
Restrictions:	The drug's efficacy and safety have to be strongly evidenced, whilst reviewing clinical trial performance.
Point of Initiation:	prescribing physician or the company
Duration of Authorization:	Defined by the AEMPS.
Required Reporting:	Efficacy and adverse events
Drug Pricing	**C**ompassionate use: No price initially, if firm will not supply for free, possibility to negotiate price centrally.
Reimbursement:	100% but only if drug is accepted for NPU
Payer:	NHS

Sweden

Legislation:	**§5 of the Medicine Act no 859 of 1992,**
Name of Programme	Named Patient Use
Responsible Government Agency	Medical Product Agency (MPA)
Restrictions:	The drug should be authorized in – EEA – An ICH country – A member country of PIC/S
Point of Initiation:	Treating physician
Duration of Authorization:	One year, with the possibility of extension
Required Reporting:	Adverse events must be reported
Drug Pricing	This is negotiated with TLV
Reimbursement:	Can be obtained via doctor's application to County Council (CC) for reimbursement.
Payer:	National Payer reimburses County Council

Switzerland

Legislation:	Therapeutic Product Act (HMG) of 15th December 2000
Name of Programme	Named Patient Use Permit(Sonderbewilligung).
Responsible Government Agency	Swiss Agency for Therapeutic Products (Swissmedic)
Restrictions:	The drug must has been previously accepted by a country.
Point of Initiation:	Treating physician
Duration of Authorization:	Fixed possible renewal
Required Reporting:	Adverse events and a final report on safety and efficacy.
Drug Pricing	Free to patient
Reimbursement:	The NPP drugs can temporarily enter in the Positive List for reimbursement / health insurance company
Payer:	Social Security System

UK

Legislation:	Schedule 1 of The Medicines for Human Use Regulations 1994
Name of Programme	Early Access to Medicines Scheme.
Responsible Government Agency	Medicines and Healthcare products Regulatory Agency (MHRA)
Restrictions:	Pharmaceutical is supplied to solve a "special need" in response to a genuine order. A scientific opinion must be made following an application to determine benefit – risk ratio.
Point of Initiation:	Treating physician
Duration of Authorization:	One year, with the possibility of extension
Required Reporting:	Adverse events must be reported
Drug Pricing	Free Pricing, provided by the sponsor, fees are attached to the submission of the programme's application.
Reimbursement:	Not Available
Payer:	Sponsor through the NHS

NOTE: At the time of writing no alteration in approach has been noted in reaction to Brexit

US

Legislation:	Schedule 1 of The Medicines for Human Use Regulations 1994
Name of Programme	Special Need. Earlier Access to Medicine Scheme.
Responsible Government Agency	Medicines and Healthcare products Regulatory Agency (MHRA)
Restrictions:	Pharmaceutical is supplied to solve a "special need" in response to a genuine order.
Point of Initiation:	Treating physician
Duration of Authorization:	One year, with the possibility of extension
Required Reporting:	Adverse events must be reported
Drug Pricing	Free Pricing
Reimbursement:	100% only via Hospital sales only; hospital budget reimbursed to NHS
Payer:	NHS Business Services Authority

About Early Access Programmes Europe

Early Access Programmes Europe

28th — 30th November, 2016- UK

Pharma IQ's Early Access Programmes Europe is the only event in Europe focused on programmes that are developed to facilitate early access to unapproved medicines. The event features 20+ speakers from pharmaceutical and biotechnology companies as well as regulatory authorities, patient advocates and HCPS.

Hear Exclusive Industry Case Studies & Benefit From Face to Face Regulatory Insight from:

- Anne B. Croppe, Vice President, Pfizer
- Daniel O'Connor, Medical Assessor, MHRA
- Arthur Caplan, Head of Bioethics, New York University Langone Medical Center
- Shekhar Natarajan, Director and Group Lead – International Regulatory Strategy, Shire Pharmaceuticals
- Julian Perez Ronco, Sr. Global Brand Medical Director, Novartis Pharma AG
- Amrit Ray, Chief Medical Officer, Johnson & Johnson

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Sources

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