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Welsh Health Specialised
Services Committee (WHSSC)

Specialised Services Policy Position Statement PP198

Cystic Fibrosis Modulator Therapies

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Policy Statement

Welsh Health Specialised Services Committee will commission Cystic Fibrosis Modulator Therapies for people with Cystic Fibrosis (CF) in accordance with the criteria outlined in this document.

In creating this document WHSSC has reviewed the relevant guidance issued by the National Institute of Health and Care Excellence (NICE)/NHS England and has concluded that these should be made available.

Disclaimer

WHSSC assumes that healthcare professionals will use their clinical judgment, knowledge and expertise when deciding whether it is appropriate to apply this policy position statement.

This policy may not be clinically appropriate for use in all situations and does not override the responsibility of healthcare professionals to make decisions appropriate to the circumstances of the individual patient, in consultation with the patient and/or their carer or guardian, or Local Authority.

WHSSC disclaims any responsibility for damages arising out of the use or non-use of this policy position statement.

1. Introduction

This Policy Position Statement has been developed for the planning and delivery of Cystic Fibrosis Modulator Therapies for people with Cystic Fibrosis (CF) resident in Wales. This service will only be commissioned by the Welsh Health Specialised Services Committee (WHSSC) and applies to residents of all seven Health Boards in Wales.

1.1 Plain Language Summary

Cystic Fibrosis is an inherited, multi-system, genetic condition that causes a build-up of sticky mucus in the lungs, digestive system and many other organs. People with CF can experience a range of problems throughout the body. In the lungs, the build-up of mucus can cause chronic infections, and in the digestive system excess mucus can cause a difficulty in digesting food.

Cystic fibrosis is caused by variants in the cystic fibrosis transmembrane conductance regulator (CFTR) gene that result in the absence or dysfunction of the CFTR protein. The CFTR protein regulates the proper flow of water and salt in and out of cells lining the lungs and other organs. This leads to the build-up of thick, sticky mucus, which can lead to infections in the lungs and damage to the pancreas. It can also lead to problems in many other parts of the body. Most people with cystic fibrosis die in early adulthood due to respiratory failure (Van Goor et al. 2014).

Many different gene variants are responsible for cystic fibrosis. The commonest variant is F508del, and approximately 50% of people with CF have two F508del genes (homozygous) and 40% have one F508del gene and another CF gene (heterozygous). Disease severity generally correlates with the severity of the loss of chloride transport.

There are approximately 10,500 people in the UK (including more than 500 in Wales) with Cystic Fibrosis¹.

1.2 The Treatments

Variants in the CFTR gene result in the absence or dysfunction of the CFTR protein, a cell-surface localised chloride channel that regulates salt and water absorption and secretion across epithelia in multiple organs. Cystic fibrosis transmembrane conductance regulator (CFTR) modulator therapies are designed to correct the malfunctioning protein by the CFTR gene. Because different variants cause different defects in the protein, the medications developed so far are effective only in people with specific gene variants. Two classes of modulators have been developed - "correctors" that facilitate processing and trafficking of the protein to the cell surface,

¹ Cystic Fibrosis strength in numbers, UK Cystic Fibrosis Registry 2020 Annual Data Report, December 2021, https://www.cysticfibrosis.org.uk/sites/default/files/2021-12/CF_Annual%20Report%202020.pdf

and “potentiators” that increase the opening ability of the channel once at the apical membrane. For some variants a potentiator alone might be enough to significantly improve ion channel function. However for Phe508del, a combination is required of a corrector to facilitate trafficking of the misfolded and prematurely degraded protein to the cell membrane, and also a potentiator to rectify the defective ion channel function when it reaches the cell membrane

There are four CFTR modulator therapies with market authorisation that act as either potentiators or correctors.

- **Ivacaftor (Kalydeco®)**
Ivacaftor is a CFTR potentiator, meaning it increases the activity of the defective CFTR protein. This means that ivacaftor increases the chances that the defective channel will open on the cell surface and let chloride and sodium ions pass through.
 - **Tezacaftor/Ivacaftor (Symkevi®)**
Ivacaftor/Tezacaftor (used in combination with Ivacaftor). Tezacaftor is a corrector designed to move the defective CFTR protein to the correct position in the cell while ivacaftor increases the activity of the defective CFTR protein.
 - **Lumacaftor/Ivacaftor (Orkambi®)**
Lumacaftor/Ivacaftor. Lumacaftor is a corrector of the CFTR working in combination with ivacaftor as a potentiator of the CFTR.
- Elexacaftor/tezacaftor/ivacaftor (Kaftrio®)**
Elexacaftor/tezacaftor/ivacaftor (used in combination with ivacaftor). Elexacaftor and tezacaftor are both correctors designed to move the defective CFTR protein to the correct position in the cell while ivacaftor increases the activity of the defective CFTR protein.

1.3 Aims and Objectives

This Policy Position Proposal aims to define the commissioning position of WHSSC on the use of CFTR modulator therapies for people with cystic fibrosis.

The objectives of this policy are to:

- ensure commissioning for the use of CFTR modulator therapies is evidence based
- ensure equitable access to CFTR modulator therapies
- define criteria for people with cystic fibrosis to access treatment
- improve outcomes for people with cystic fibrosis.

1.4 What NHS Wales has decided

The Welsh Health Specialised Services Committee has carefully reviewed the guidance issued by NICE and NHS England and have confirmed that there is enough evidence to recommend that ivacaftor, tezacaftor/ivacaftor, lumacaftor/ivacaftor and elexacaftor/tezacaftor be made available as treatment options for people with cystic fibrosis who have one of an expanded range of cystic fibrosis transmembrane conductance regulator (CFTR) variants within the criteria set out in Section 2.1.

2. Criteria for Commissioning

The Welsh Health Specialised Services Committee approve funding of the following CFTR modulator therapies for patients with CF in-line with the criteria identified in this policy:

- ivacaftor
- lumacaftor/ivacaftor
- tezacaftor/ivacaftor
- elexacaftor/tezacaftor/ivacaftor

These medications will be available to people with variants approved and licensed by the [Medicines and Healthcare Products Agency \(MHRA\)](#) in addition to those named variants approved and licenced by the [US Food and Drug Administration \(FDA\)](#) for which the use of medicines would be off-label.

2.1 Licenced Inclusion Criteria

- Ivacaftor for people who are aged 4 months and above for the R117H variant and for named gating variants when heterozygous in the CFTR gene.
- Lumacaftor/ivacaftor for people who are aged 2 years and older who are homozygous for the F508del variant in the CFTR gene.
- Tezacaftor/ivacaftor for the treatment of people with CF aged 6 years and older who are homozygous for the F508del variant or heterozygous in the CFTR gene for any one of 14 variants combined with F508del variants.
- Elexacaftor/tezacaftor/ivacaftor for people aged 6 years and older who have two F508del variants or one F508del in combination with any variant.

The look up table of licenced variants from the manufacturer can be found at [Vertex Treatments Finder | CFSource](#)

For all these CFTR modulator products where the UK license is amended in the future, eligible patients will automatically have access under those terms. The manufacturer will be responsible for supplying age-appropriate products within Europe.

2.2 Off-label use

A clinician considering prescribing a medication outside of the terms of the licence (off-label) should do so in accordance with the [Medicines and Healthcare Products Agency \(MHRA\)](#) and the [General Medical Council \(GMC\)](#) guidance which applies throughout the UK.

The risks and benefits of off-label use of all the modulator therapies should be clearly stated and discussed with the patient to enable informed consent.

The [GMC guidance](#) states prescribing unlicensed medicines may be necessary where “there is no suitably licenced medicine that will meet the patients need”. Should clinicians consider this appropriate for their patients and they have followed local medicines governance arrangements for off-label use, WHSSC will meet the cost for those variants as listed in section 2.2.1.

2.2.1 Named CFTR variants that will not be considered by the UK regulator

Where the UK medicines regulator (MHRA) has not yet considered the evidence for some named CFTR variants, WHSSC will reimburse the off-label use of the following therapies in line with the approach to in vitro data taken by the US Food and Drug Administration (FDA) for the approved named variants:

- Ivacaftor: People with CF aged 4 months and older who are heterozygous in the CFTR gene for any one of 97 named variants outside of the licence.
- Tezacaftor/ivacaftor: People with CF aged 6 and older who have any one of 140 named variants outside the UK licence. In addition, 14 variants licenced by the UK regulator for people with CF who have the F508del variant are included for off-label prescribing when combined with any variant other than F508del.
- Elexacaftor/tezacaftor/ivacaftor: For people with CF aged 6 and older who are heterozygous for any one of the 177 named variants outside of the UK licence which can be combined with any other variant.

2.3 Prescribing Guidance and Monitoring Criteria

2.3.1 Prescribing

The CFTR therapies should only be prescribed in line with this commissioning policy and by physicians with experience in the treatment of cystic fibrosis working in commissioned NHS Wales and NHS England cystic fibrosis services.

For people whose genotype is unknown, an accurate and validated genotyping method should be performed before starting treatment to confirm the presence of an indicated variant in the CFTR gene (see ivacaftor, tezacaftor/ivacaftor and elexacaftor/tezacaftor/ivacaftor below).

Cystic Fibrosis teams will need to review existing patients prior to changing or initiating new medications.

Moderate transaminase (alanine transaminase (ALT) or aspartate transaminase (AST)) elevations are common in people with CF. Liver function tests will be done for all people prior to initiating ivacaftor either in monotherapy or in a combination regime as tezacaftor/ivacaftor or as the triple therapy elexacaftor/tezacaftor/ivacaftor.

As ivacaftor contains lactose, ivacaftor in either monotherapy or in the combination regimen as Tezacaftor/ivacaftor or elexacaftor/tezacaftor/ivacaftor should not be prescribed to people with rare hereditary problems of galactose intolerance, total lactose deficiency or congenital glucose-galactose malabsorption.

2.3.2 Ivacaftor

Treatment with ivacaftor as a monotherapy is available to adults, adolescents and children aged 4 months and older with CF who have at least one copy of 97 named variants in the CFTR gene. The other CF variant can be any variant.

Eleven of the 97 variants² are marked as being a 'variant of varying clinical consequence'³ (VVCC)⁴. It is therefore important that supportive diagnostic criteria are used in addition to the presence of the variant. In these cases, a definitive CF diagnosis requires sweat chloride >60 mmol/L abnormal nasal potential difference or abnormal intestinal current measurement on rectal biopsy⁵.

² D74W, S977F, R1070Q, D1152H, D110E, F1052V, R1070W, D1270N, D579G, G1069R, F1074L

³ This means that some people with this gene change, combined with another CF causing variant, have CF. Other people with this gene change combined with another CF causing variant, do not have CF.

⁴ Clinical and Functional Translation of CFTR. <https://www.cftr2.org>

⁵ Clinical and Functional Translation of CFTR, <https://www.cftr2.org>

Ivacaftor dosage

Table 1: Dose recommendations for infants aged at least 4 months, toddlers and children*

Age	Weight	Dose	Total daily dose
4 months to less than 6 months	≥5 kg to < 7 kg	25 mg granules taken orally every 12 hours with fat-containing food	50 mg
6 months and older	≥5 kg to < 7 kg	25 mg granules taken orally every 12 hours with fat-containing food	50 mg
	≥ 7 kg to < 14 kg	50mg granules taken orally every 12 hours with fat-containing food	100 mg
	≥ 14 kg to < 25 kg	75 mg granules taken orally every 12 hours with fat-containing food	150 mg
	>25 kg	See Summary of Product Characteristics (SmPC) (see section 2.3.3).	

*The formulation to be administered for patients weighing <25kg is granules. The required dose should be mixed with 1 teaspoon (5mls) of soft food.

Table 2: Dose recommendations for adults and paediatric patients aged 6 and older and weighing more than 25kg

Weight	Dose
Patients ≥ 25kg	One 150mg tablet is taken orally every 12 hours with fat- containing food

Clinicians should refer to the current Summary of Product Characteristics (SmPC) (see [section 2.3.3](#)) before prescribing, and for dose modification for patients with co-morbidities and those taking concomitant medicines. The dose of ivacaftor should be adjusted when co-administrated with moderate and strong CYP3A inhibitors.

Tezacaftor/ivacaftor dosage

Table 3: Tezacaftor/ivacaftor dosing recommendations for patients aged 6 years and older*

Weight	Morning	Evening
<30 kg	50 mg tezacaftor and 75 mg ivacaftor taken orally every 12 hours with fat-containing food	75 mg ivacaftor
≥ 30 kg	100 mg tezacaftor and 150 mg ivacaftor taken orally every 12 hours with fat-containing food	150 mg ivacaftor
≥ 12 years	100 mg tezacaftor and 150 mg ivacaftor taken orally every 12 hours with fat-containing food	150 mg ivacaftor

*The recommended dose should be taken in the morning and evening, approximately 12 hours apart with fat-containing food

The dose of tezacaftor/ivacaftor and ivacaftor should be adjusted when co-administered with moderate and strong CYP3A inhibitors or if the patient has hepatic impairment, as described in the SmPC (see [section 2.3.3](#)).

Lumacaftor/ivacaftor as a combination therapy dosage

Table 4: Lumacaftor/ivacaftor as a combination therapy: for patients aged 2 years and over*

Age	Dose
2 to 5 years and weighing 14 kg or greater	Two lumacaftor 150 mg/ivacaftor 188 mg tablets every 12 hours
2 to 5 years and weighing less than 14 kg	One lumacaftor 100 mg/ivacaftor 125 mg sachet every 12 hours
6 to 11 years	Two lumacaftor 100 mg/ivacaftor 125 mg tablets every 12 hours
≥ 12 years and older	Two lumacaftor 200 mg/ivacaftor 125 mg tablets every 12 hours

* The recommended dose should be taken in the morning and evening, approximately 12 hours apart with fat-containing food

Clinicians should refer to the current SmPC (see [section 2.3.3](#)) before prescribing and for dose modifications if patients are on other therapies or have co-morbidities.

Elexacaftor/tezacaftor/ivacaftor dosage

Table 5: Elexacaftor/tezacaftor/ivacaftor dosing for patients aged 6 years and older*

Age	Morning	Evening
6-12yrs and <30kg	Two tablets (each containing elexacaftor 50mg, tezacaftor 25mg and ivacaftor 37.5mg)	One tablet of 75mg ivacaftor
6-12yrs and ≥30kg	Two tablets (each containing elexacaftor 100 mg, tezacaftor 50 mg and ivacaftor 75 mg)	One tablet of 150 mg ivacaftor
≥ 12 years	Two tablets (each containing elexacaftor 100 mg, tezacaftor 50 mg and ivacaftor 75 mg)	One tablets of 150 mg ivacaftor

*For people aged 6 years and above the recommended dose is in the form of oral tablets swallowed whole and taken in the morning and evening, approximately 12 hours apart with fat-containing food.

The dose of elexacaftor/tezacaftor/ivacaftor should be adjusted when co-administered with moderate and strong CYP3A inhibitors or if the patients has hepatic impairment as described in the SmPC (see [section 2.3.3](#)).

2.3.3 Summary of Product Characteristics (SmPC)

The marketing authorities for each product cover side effects, contraindications, drug interactions, and the need to consider variation in dosing when ivacaftor is given in combination with other products as well as age and weight specific dosing.

Summaries of Product Characteristics (SmPC) are available from [The Electronic Medicines Compendium \(EMC\)](#) for the following:

- [Ivacaftor \(Kalydeco®\)](#)
- [Ivacaftor/lumacaftor \(Orkambi®\)](#)
- [Ivacaftor/tezacaftor \(Symkevi®\): Used in combination with ivacaftor](#)
- [Elexacaftor/tezacaftor/ivacaftor \(Kaftrio®\): tablets used in combination with ivacaftor.](#)

2.3.4 Monitoring Criteria

Where the benefits of testing outweighs the risks of potential exposure to COVID-19, liver function tests and blood pressure monitoring will be done at least every 3 months during the first year of treatment and annually thereafter for all patients taking ivacaftor treatment, either in monotherapy, in a combination regimen with lumacaftor, tezacaftor/ivacaftor or as triple therapy elexacaftor/tezacaftor/ivacaftor⁶.

In line with guidance from the Royal College of Ophthalmologists⁷ it is recommended that when paediatric patients are starting ivacaftor treatment, either in monotherapy, in a combination regimen with lumacaftor, tezacaftor/ivacaftor or as the triple therapy, elexacaftor/tezacaftor/Ivacaftor, they should be seen on a regular basis by their local optometrist to detect any significant visual difficulties which may prompt referral to hospital eye services for further assessment.

2.4 Stopping Criteria

In the event of significant elevations of transaminases (e.g. patients with ALT or AST > 5 x the upper limit of normal or ALT or AST >3 x upper limit of normal with bilirubin >2 x upper limit of normal) dosing with ivacaftor, tezacaftor/ivacaftor or elexacaftor/tezacaftor/ivacaftor should be interrupted and laboratory tests closely followed until abnormalities resolve.

Consideration should be given to delaying or discontinuing therapy if hepatotoxicity or renal toxicity occurs.

During pregnancy it is preferable to avoid the use of ivacaftor, tezacaftor/ivacaftor or elexacaftor/tezacaftor/ivacaftor. For women who are breast feeding and taking ivacaftor, tezacaftor/ivacaftor or elexacaftor/tezacaftor/ivacaftor, a decision should be made whether to discontinue breast feeding or discontinue/abstain from ivacaftor, tezacaftor/ivacaftor or elexacaftor/tezacaftor/ivacaftor, taking into account the benefit of breast feeding for the child and the benefit of therapy for the woman.

⁶ Covid-19 rapid guideline: Cystic Fibrosis. NICE 2020. <https://nice.org.uk/guidance/ng170/chapter/3-Modifications-to-usual-care-and-service-delivery>

⁷ Advice for clinicians on Cataract Related to CFTR Modifying Drug of Cystic Fibrosis. The Royal College of Ophthalmologists 2019. <https://www.rcophth.ac.uk/wp-content/uploads/2019/12/Cataract-related-CFTR-for-cystic-fibrosis.pdf>.

2.5 Continuation of Treatment

Healthcare professions are expected to review a patient's health record at regular intervals to ensure that they are demonstrating an improvement to their health due to the treatment being given.

If no improvement to a patient's health has been recorded, then clinical judgement on the continuation of treatment should be made by the treating healthcare professional.

2.6 Acceptance Criteria

The service outlined in this specification is for patients ordinarily resident in Wales, or otherwise the commissioning responsibility of the NHS in Wales. This excludes patients who whilst resident in Wales, are registered with a GP practice in England, but includes patients resident in England who are registered with a GP Practice in Wales.

2.7 Designated Centre

Adult and Young people in South Wales have access to:

The All Wales Cystic Fibrosis Centre based at:

- University Hospital Llandough
Penlan Road
Llandough
CF64 2XX

People also have access to:

- Bristol Royal Infirmary
Marlborough Street
Bristol
BS2 8HW
- Royal Brompton Hospital
Sydney Street
Chelsea
London
SW3 6NP

Adult and Young People in North Wales and Powys have access to:

- Liverpool Heart and Chest Hospital.
Thomas Drive
Liverpool
L14 3PE

West Midlands Adult Cystic Fibrosis Centre at:

- Birmingham Heartlands Hospital
Bordesley Green East
Birmingham
B9 5SS

Manchester Adult Cystic Fibrosis Centre at:

- Wythenshawe Hospital
Manchester Adult CF Centre
South Moor Road
Manchester
M23 9LT
- Royal Stoke University Hospital
Newcastle Road
Stoke-on-Trent
ST4 6QG

Infants, Toddlers and children have access to:

- The Children's Hospital for Wales
Heath Park
Cardiff
CF14 4XW
- Alder Hey Children's Hospital
E Prescott Road
Liverpool
L14 5AB
- Birmingham Children's Hospital
Steelhouse Lane
Birmingham
B4 6NH
- Royal Manchester Children's Hospital
Oxford Road
Manchester
M13 9WL

2.8 Exceptions

If the patient does not meet the criteria for treatment as outlined in this policy, an Individual Patient Funding Request (IPFR) can be submitted for consideration in line with the All Wales Policy: Making Decisions on Individual Patient Funding Requests. The request will then be considered by the All Wales IPFR Panel.

If the patient wishes to be referred to a provider outside of the agreed pathway, and IPFR should be submitted.

Further information on making IPFR requests can be found at: [Welsh Health Specialised Services Committee \(WHSSC\) | Individual Patient Funding Requests](#).

2.9 Clinical Outcomes and Quality Measures

The Provider should work to written quality standards and provide monitoring information to the lead commissioner and the CF Registry.

[NICE provide a data collection agreement](#) to include the therapies within this policy position statement. Health Boards and Trusts currently submit data on the numbers of patients treated with CFTR modulators to the national [Cystic Fibrosis Registry](#) which is hosted by the Cystic Fibrosis Trust.

All providers should submit the minimum dataset data to the UK Cystic Fibrosis (CF) Registry within the required timescales. There is an expectation that data from all of Welsh CF patients will be entered onto the CF registry, but where it is not possible to obtain consent for registration and data entry the reasons should be documented for future audit.

Data collection as part of the access agreement will be used by NICE to inform further evaluation of Lumacaftor/Ivacaftor and to support a clinical and cost effectiveness evaluation of Tezacaftor/Ivacaftor.

NICE will extend data collection to support a clinical and cost effectiveness evaluation of elexacaftor/tezacaftor/ivacaftor.

The centre should enable the patient's, carer's and advocate's informed participation and to be able to demonstrate this. Provision should be made for patients with communication difficulties and for children, teenagers and young adults.

2.10 Responsibilities

Referrers should:

- inform the patient that this treatment is not routinely funded outside the criteria in this policy, and
- refer via the agreed pathway.

Clinician considering treatment should:

- discuss all the alternative treatment with the patient
- advise the patient of any side effects and risks of the potential treatment

- inform the patient that treatment is not routinely funded outside of the criteria in the policy, and
- confirm that there is contractual agreement with WHSSC for the treatment.

In all other circumstances an IPFR should be submitted.

3. Documents which have informed this policy

The following documents have been used to inform this policy:

- **NHS England policies**

- Updated Commissioning Statement: Ivacaftor, tezacaftor/ivacaftor, lumacaftor/ivacaftor and elexacaftor/tezacaftor/ivacaftor for licensed and off-label use in patients with cystic fibrosis who have named variants. [Publication reference:210508P, January 2022.](#)

This document should be read in conjunction with the following documents:

- **NHS Wales**

- All Wales Policy: [Making Decisions in Individual Patient Funding requests](#) (IPFR).

4. Date of Review

This document will be reviewed when information is received which indicates that the policy requires revision.

5. Putting Things Right

5.1 Raising a Concern

Whilst every effort has been made to ensure that decisions made under this policy are robust and appropriate for the patient group, it is acknowledged that there may be occasions when the patient or their representative are not happy with decisions made or the treatment provided.

The patient or their representative should be guided by the clinician, or the member of NHS staff with whom the concern is raised, to the appropriate arrangements for management of their concern.

If a patient or their representative is unhappy with the care provided during the treatment or the clinical decision to withdraw treatment provided under this policy, the patient and/or their representative should be guided to the LHB for [NHS Putting Things Right](#). For services provided outside NHS Wales the patient or their representative should be guided to the [NHS Trust Concerns Procedure](#), with a copy of the concern being sent to WHSSC.

5.2 Individual Patient Funding Request (IPFR)

If the patient does not meet the criteria for treatment as outlined in this policy, an Individual Patient Funding Request (IPFR) can be submitted for consideration in line with the All Wales Policy: Making Decisions on Individual Patient Funding Requests. The request will then be considered by the All Wales IPFR Panel.

If an IPFR is declined by the Panel, a patient and/or their NHS clinician has the right to request information about how the decision was reached. If the patient and their NHS clinician feel the process has not been followed in accordance with this policy, arrangements can be made for an independent review of the process to be undertaken by the patient's Local Health Board. The ground for the review, which are detailed in the All Wales Policy: Making Decisions on Individual Patient Funding Requests (IPFR), must be clearly stated

If the patient wishes to be referred to a provider outside of the agreed pathway, an IPFR should be submitted.

Further information on making IPFR requests can be found at: [Welsh Health Specialised Services Committee \(WHSSC\) | Individual Patient Funding Requests](#)

6. Equality Impact and Assessment

The Equality Impact Assessment (EQIA) process has been developed to help promote fair and equal treatment in the delivery of health services. It aims to enable Welsh Health Specialised Services Committee to identify and eliminate detrimental treatment caused by the adverse impact of health service policies upon groups and individuals for reasons of race, gender re-assignment, disability, sex, sexual orientation, age, religion and belief, marriage and civil partnership, pregnancy and maternity and language (Welsh).

This policy has been subjected to an Equality Impact Assessment.

The Assessment demonstrates the policy is robust and there is no potential for discrimination or adverse impact. All opportunities to promote equality have been taken.

Annex i Abbreviations and Glossary

Abbreviations

IPFR	Individual Patient Funding Request
WHSSC	Welsh Health Specialised Services
CFTR	Cystic Fibrosis Transmembrane Conductor Regulator

Glossary

Individual Patient Funding Request (IPFR)

An IPFR is a request to Welsh Health Specialised Services Committee (WHSSC) to fund an intervention, device or treatment for patients that fall outside the range of services and treatments routinely provided across Wales.

Welsh Health Specialised Services Committee (WHSSC)

WHSSC is a joint committee of the seven local health boards in Wales. The purpose of WHSSC is to ensure that the population of Wales has fair and equitable access to the full range of Specialised Services and Tertiary Services. WHSSC ensures that specialised services are commissioned from providers that have the appropriate experience and expertise. They ensure that these providers are able to provide a robust, high quality and sustainable services, which are safe for patients and are cost effective for NHS Wales.

CFTR gene

Refers to the cystic fibrosis transmembrane conductance regulator (CFTR) gene which contains the instructions for making CFTR protein.

Variant

In this context variant refers to the changing of the structure of a gene, resulting in a variant form that may be transmitted to subsequent generations.

In-vitro Study

A study performed or taking place in a test tube, culture dish or elsewhere outside a living organism.