

# Guide to: Umbrella Access Programs (UAP)

A Pioneering,  
Multi-Stakeholder  
Approach to Early Access

# What is Early Access?

Regulatory route allowing access to a product whether it is globally-unlicensed or not licensed/commercially-available in its country of intended use

# Who is Early Access for?

## **The criteria usually specify patients:**

- who have a serious or life-threatening disease
- have exhausted all commercially-available treatment options, and are unable to enroll in a clinical trial

## **Intention is primarily to provide treatment:**

- prior to regulatory approval
- to bridge gap between trial completion/filing and launch, or in countries where the product will never be launched
- most countries have legislation to facilitate such access

Case example



**HAEi**  
HAE International

HAE International is a global non-profit network of member organizations dedicated to raising awareness of hereditary angioedema (HAE) and improving the lives of people with HAE



# HAEi Global Access Program

Provides life-saving medication to HAE patients around the world

“

The pioneering HAEi GAP is the first instance of a 3-way access program partnership, bringing benefits to all parties, and supporting patients around the world through increased health equity”



**STUART BELL**

Senior Vice President  
Inceptua Early Access



**HENRIK  
BALLE BOYSEN**  
Executive Vice President  
HAEi

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Access to medication is key to any patient suffering from a life-threatening disease like HAE.

Our partnership with Inceptua on the HAEi Global Access Program provides an opportunity and a mechanism for physicians to access modern HAE medication for their patients”

# The UAP principles I

- One visible/official source for the provision of multiple treatment options (unavailable in the country of intended use at that time point) from multiple manufacturers, for a specific disease
- Utilizes compliant unlicensed medicines regulatory pathways
- Treatments can either be globally-unlicensed, licensed in 1 or some territories (as commercial rollout progresses), or available in all countries where commercialization will occur



# The UAP principles II

- Under all 3 scenarios, demand exists for access to promising treatments, not available in their country of intended use
- Ideally in a partnership with a disease-specific patient organization to support broader visibility and greater patient equity
- There is no 'competition' between participating companies, as all requests are product-specific, many products do not overlap entirely by label, and specialist physicians working in a particular rare disease are likely to have multiple patients, eligible for different products

# How it works?

- Each participating company contracts separately with Inceptua
- Products are made available via Inceptua's custom-built online platform (IMAP), supported by Inceptua's customer service team for regulatory support through the ordering process
- Inceptua's IMAP portal supports outcomes data collection from patients on the UAP
- Inceptua customer service team are product agnostic – they make no recommendation for products and merely respond to a specific request from a physician for access to a particular product

# Key Benefits I

- Reduce access inequity, by extending the geography of product distribution, beyond the scope of commercialized countries, or through allowing access prior to launch in a specific country
- Satisfy physician and patient demand
- Ethical imperative, goodwill amongst prescriber/patient communities, positive CSR
- One visible resource for physicians, making it easier for them to source treatments for their patients with no suitable treatment option

# Key Benefits II

- Allows the collection of data from a wider population of patients, reflective of the real-world setting
- Umbrella Access Programs operate within a non-competitive space, and the more companies who participate in a UAP the more significant the broader benefits i.e. the more momentum and visibility the UAP gains, and the greater number of physicians/patients who benefit

# What disease areas?

- The UAP concept is suited to many different diseases
- Particularly diseases characterized by high unmet need, like rare diseases
- Where multiple products are approved for the same indication, but where company commercialization footprint varies, there will be demand in certain countries which UAPs can support
- Patient group involvement supports awareness



**INCEPTUA**  
EARLY ACCESS

# Any competitive risks?

- No. Physicians contact Inceptua to request a specific medicine for a specific patient
- Inceptua does not inform enquiring physicians of other products available in the same UAP
- Inceptua's customer service team merely support requesting physicians through the process of ordering whichever product they request

# Any competitive risks?

- Even where multiple products exist for the same indication, often these will differ in where they fall in the treatment paradigm, they may vary by patient sub-populations, by mechanism of action, route of administration, current geographic scope of availability etc.
- Any physician specializing in a particular disease area may well have many patients who may benefit from different products within a UAP

# Any competitive risks?

- Although the UAP is presented as one visible resource for physicians to access medicines for a particular indication, it is run as multiple, distinct programs, so physicians will only be able to see the specifics of the product they are seeking for a particular patient



# Inceptua Early Access

Experts in the strategy,  
design and implementation  
of global early access  
programs

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