Challenges in drug development, regulation and clinical practice for

immunoglobulins

IPFA perspectives on the clinical development and clinical use of Ig

on behalf of IPFA by

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Conflicts of interest
Karen Pinachyan

- Current employee: LFB
- Former employment and shareholder: CSL Behring
- Former employment: Octapharma

The views expressed are those of the presenter and should not be understood or quoted as being made on behalf of LFB

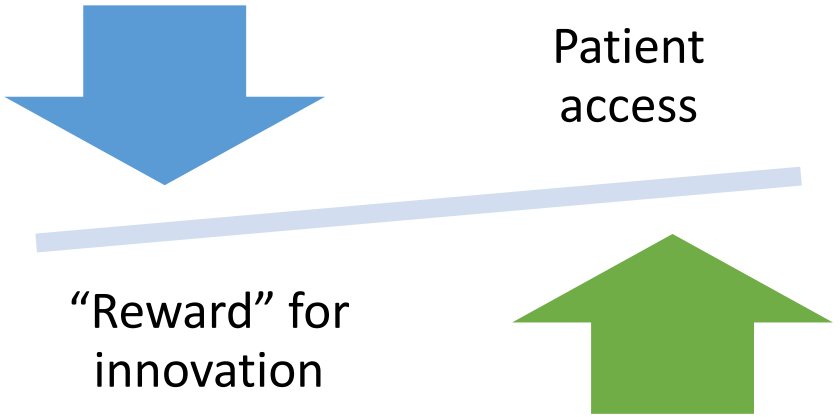
LFB
ETHICAL COMMITMENT

IPFA, the association for the not-for-profit plasma organisations

- The International Plasma and Fractionation Association (IPFA) is an association representing organisations engaged in *fractionation of plasma into plasma-derived medicines as well as in the collection or testing of plasma for fractionation purpose*.
- IPFA´s members are from the not-for-profit sector and include organisations with a clear public mission.
- Members come from all over the world and represent
  - manufacturers (fractionators) who produce the plasma products,
  - blood establishments collecting plasma
  - institutions who carry out clinical quality testing of plasma.

Dynamic balance

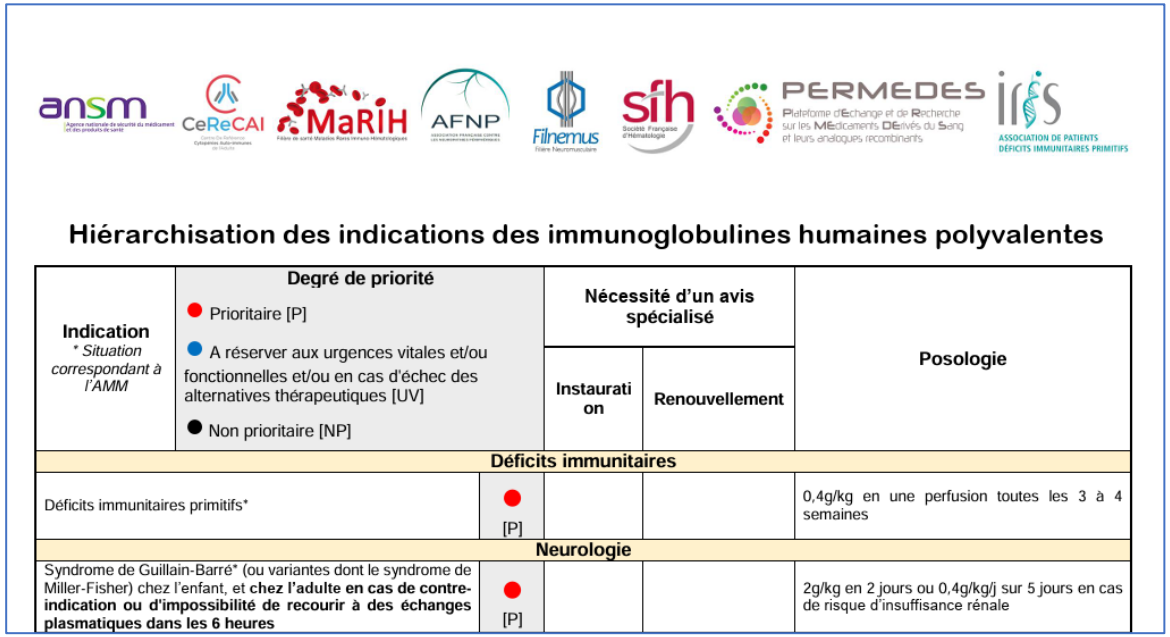
Image created by DALL-E (OpenAI), prompted to visualise the dynamic balance between reward for innovation and patient access to treatment



In a moment of need… prioritisation exercise

Based on :

- Therapeutic alternatives available
- Quality of Evidence available
- Other considerations



Hiérarchisation des indications des immunoglobulines humaines polyvalentes

Indication ** Situation correspondant à l'AMM*	Degré de priorité ● Prioritaire [P] ● A réserver aux urgences vitales et/ou fonctionnelles et/ou en cas d'échec des alternatives thérapeutiques [UV] ● Non prioritaire [NP]	Nécessité d'un avis spécialisé		Posologie
		Instauration	Renouvellement	
Déficits immunitaires				
Déficits immunitaires primitifs*	● [P]			0,4g/kg en une perfusion toutes les 3 à 4 semaines
Neurologie				
Syndrome de Guillain-Barré* (ou variantes dont le syndrome de Miller-Fisher) chez l'enfant, et **chez l'adulte en cas de contre-indication ou d'impossibilité de recourir à des échanges plasmatiques dans les 6 heures**	● [P]			2g/kg en 2 jours ou 0,4g/kg/j sur 5 jours en cas de risque d'insuffisance rénale

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Clinical commissioning policy for the use of therapeutic immunoglobulin (Ig) England (2024)

Document first published: 9 December 2021
Page updated: 29 April 2024
Topic: Commissioning
Publication type: Policy or strategy

Documents relating to the updated commissioning criteria policy for the use of therapeutic Immunoglobulin (Ig) in England.

Document

.pdf

Clinical commissioning policy for the use of therapeutic immunoglobulin (Ig) England (2024)

PDF 703 KB 36 pages

Summary

Therapeutic immunoglobulin is recommended to be available as a routinely commissioned treatment option for the indications within the criteria set out in these documents.

Document

.pdf

Change form: Clinical commissioning policy for the use of therapeutic immunoglobulin (Ig) England, 2021

PDF 281 KB 21 pages

Summary

The Commissioning criteria policy for the use of therapeutic immunoglobulin (Ig) England, 2021, has been converted into a policy template with an update of the document following publication. These changes were agreed previously (v1.0 – unpublished) and do not form part of this change form.

Document

.pdf

Clinical Priorities Advisory Group policy update: recommendations on therapeutic Immunoglobulin

PDF 53 KB 4 pages

Summary

Outline of the criteria required by the the Clinical Priorities Advisory Group (CPAG) for the commissioning criteria policy for the use of therapeutic Immunoglobulin.

Challenges and Opportunities in the dynamic balance

- Very costly clinical studies (including the IMP very high costs), in a situation of regular supply challenge
- Rare diseases / over-solicited patient population / existing approved “use” (in-label or “guideline-recommended”)
- Country-level Market Access process not considering the available evidence for price differentiation
- Evolving pathology / treatment landscape (especially in Secondary Immunodeficiency, eg arrival of CAR-T-related use of immunoglobulins)
- Existence of core SmPC / Ig’s are not interchangeable and not biosimilars
- Plasma collection geographical imbalance and Ig supply/demand evolution

How can we overcome challenges together?

- Predictability/Clarity/Transparency around Core SmPC process and its evolution
- Collaborative approach to clinical development, including anticipation of potential iatrogenic side effects of other drugs
- Innovative approach to evidence generation and consideration & use of all available evidence
- Use of real-world evidence to clarify/confirm Core SmPC indications / provide clinical use guidance (eg dose)

Clinical Evidence 2030

Peter Arlett[1], Denise Umuhire[1,*], Patrice Verpillat[1], Paolo Foggi[2], Ulla Wändel Liminga[3], Bruno Sepodes[4], Marianne Lunzer[5], Brian Aylward[6], Spiros Vamvakas[1], Kit Roes[7], Frank Pétavy[1], Steffen Thirstrup[1], Maria Lamas[8], Emer Cooke[1] and Karl Broich[9]

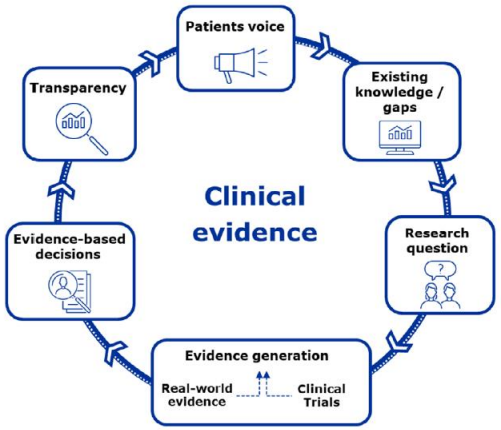

Figure 1 Representation of the vision for clinical evidence 2030.

PRINCIPLE 5: THE GENERATION OF CLINICAL EVIDENCE IS PLANNED EARLIER AND COLLABORATIVELY ACROSS HEALTHCARE STAKEHOLDERS, ALLOWING THEM TO FULLY LEVERAGE THE TOTALITY OF EVIDENCE

PRINCIPLE 4: THE FULL SPECTRUM OF DATA AND METHODS IS EMBRACED, WITH BETTER, SMARTER, AND FASTER CLINICAL TRIALS REMAINING AT THE CORE OF CLINICAL EVIDENCE, COMPLEMENTED BY REAL-WORLD EVIDENCE FOR WHICH EVIDENTIARY VALUE IS ESTABLISHED ACROSS THE FULL SPECTRUM OF RESEARCH QUESTIONS

Thank you !

Any feedback?
Reach out !

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Special thanks to IPFA team (Françoise Rossi, Leni von Bonsdorff)
& colleagues who provided ideas, insights and feedback for the presentation