

La metodologia per “unmet clinical needs”

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*Una nuova storia: Il Tagraxofusp e la BPDCN.
Bologna 2 maggio 2023*

Guideline Article - Expert opinion

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Unmet Clinical Needs and Management Recommendations for Blastic Plasmacytoid Dendritic Cell Neoplasm: A Consensus-based Position Paper From an Ad Hoc International Expert Panel

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Unmet clinical needs: the health technology assessment (HTA) view

Unmet clinical need is an area where to invest in research with the final aim to develop a new technology (drug, device) that can have a place in the market

Unmet clinical needs: the “scientific community “ view

Unmet clinical needs are patient-directed knowledge/decisional uncertainties whose solution is relevant for the patient outcome (cure, survival, quality of life)

Clinical needs - categories

- Diagnostic needs
- Prognostic needs
- Therapeutic needs
- Communication needs
- Research needs

Identifying and addressing unmet clinical needs in the management of BPDCN: a consensus – based proposal from an ad hoc expert panel

The objectives of this project were to:

- **Identify the most clinically relevant decisional uncertainties (UCNs)** in the management (diagnosis, staging, therapy) of blastic plasmacytoid dendritic cell neoplasm (BPDCN)
- **Produce practice recommendations** concerning the identified clinical needs with sufficient knowledge (experimental evidence, real world evidence, practice)
- **Suggest operational solutions (proposals)** for unsolvable unmet clinical needs, i.e. propose actionable research for improving knowledge

Identifying and addressing unmet clinical needs in the management of BPDCN: a consensus – based proposal from an ad hoc expert panel

Steps in the projects:

- **Select an expert panel**
- Identify unmet clinical needs (**Delphi technique**)
- **Prioritize the most clinically relevant clinical needs** (group discussion)
- Consensus on the proposed solutions (**Delphi technique and nominal group technique**)

Unmet clinical needs selected by the Panel

- Optimization of subclassification: pediatric versus adult
- Optimization of the prognostic stratification
- Indication to allotransplant
- Indication to autotransplant
- Optimization of the staging pathway
- Optimization of the diagnostic pathway
- CNS prophylaxis
- Multidisciplinary management coordination
- Making the pediatric groups more aware of the disease
- Optimization of subclassification: plasmacytoid vs. AXL+ dendritic cell neoplasms
- Therapeutic recommendations for young (and fit) patients
- Mechanisms of drug resistance
- Therapeutic recommendations for elderly or unfit patients

Outline of the project

Sponsor/funder: Menarini

Scientific coordination: Livio Pagano, Stefano Pileri, Pierluigi Zinzani,

Methodological coordination: Giovanni Barosi

Expert Panel

1. Emilio Berti (Milano, Italy)
2. Hagop Kantarjian (MD Anderson, USA)
3. Francesco Onida (Milano, Italy)
4. Alberto Orfao (Salamanca, Spain)
5. Livio Pagano (Rome, Italy)
6. Stefano A. Pileri (Milano, Italy)
7. Reim Willemze (Leiden, Netherland)
8. Pierluigi Zinzani (Bologna)