Access to treatment

in PH groups 1 & 4 (PAH & CTEPH)

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Access to PH treatment – main determinants

• Awareness
  - among medical staff and populations at risk

• Reliable differential diagnosis
  - to identify those with PAH and CTEPH among millions of PH patients

• Local availability of medical and interventional Tx
  - access to approved drugs and validated interventions - unrestricted by cost
NEW ALGORITHM FOR DIAGNOSIS OF PULMONARY HYPERTENSION AND ITS CAUSES:
Triage of urgent cases and diagnosis of common conditions

ASSESS PROBABILITY OF PH

IDENTIFY HIGH RISK PATIENTS

DIAGNOSE COMMON CAUSES OF PH

DIAGNOSE RARE CAUSES OF PH

CTEPH, chronic thromboembolic pulmonary hypertension
PAH, pulmonary arterial hypertension
PH, pulmonary hypertension; SPECT, single-photon emission computed tomography
V/Q, nuclear ventilation perfusion scan

Footnotes:
1. Described in ESC/ERS guidelines 2013
2. These include chronic thromboembolic disease without pulmonary hypertension which should be considered in patients with risk factors and / or previous venous thromboembolism
3. SPECT or planar V/Q scan is acceptable. Interpretation is binary
4. Referral to be seen in person or for a teleconsultation
NEW ALGORITHM FOR DIAGNOSIS OF PULMONARY HYPERTENSION AND ITS CAUSES:
Triage of urgent cases and diagnosis of common conditions

Asses probability of PH

Identify patients at high risk of PAH/CTEPH

Direct pts with common PH, group 2-3, to regular HCP

Refer pts suspected of PAH/CTEPH or unclear severe PH to dedicated Expert Centres

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ERN-LUNG: Patient Pathway via Core Networks for specific diagnosing and Cross Border Care

Patient with unclear respiratory disease referred by tertiary centre (e.g. local, regional, national referral/Supporting Partner referral) sent to presumed specific ERN-LUNG Core Network

Level I: EXpert Advisory BOard Online Consultation *
- Question is forwarded online to Medical Expert
- Within the Core Network or across different Core Networks

Adequate treatment identification and performance *
- If no satisfying solution has been found, e.g.:
  a) classification within Core Network not certain
  b) no adequate treatment could be identified
  c) further expert opinion required (e.g. by additional medical area)

Enter Cross Border Care Pathway:

Level II: Cross Border Online Consultation via CPMS *
- CPMS Panel discussion
- Panel composition on demand

Level III: Cross Border Referral of Patient
- If all preconditions (see left) have been met
- The leading physician caring for the patient is endorsed by ERN-LUNG to state that the patient needs cross-border referral

* As soon as satisfying solution identified, pathway will be left and patient referred either to a dedicated HCP-CN Member or to a non-ERN clinical centre at a national level
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- **EXABO (nat.)**
  - EXABO (cb)

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## Competency Requirements:
### Minimum patient numbers & procedures

<table>
<thead>
<tr>
<th>Indicator</th>
<th>Minimum # required / HCP / year</th>
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</thead>
<tbody>
<tr>
<td>Minimum number of TOTAL patients (visited, treated or followed) per year</td>
<td>Adult 200, Children 30</td>
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<tr>
<td>Minimum number of NEW patients per year</td>
<td>Adult 50, Children 10</td>
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<tr>
<td>Key diagnostic procedures per year</td>
<td></td>
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<tr>
<td>Acute vasoreactivity challenge</td>
<td>Adult 20, Children 10</td>
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<tr>
<td>Right heart catheterization</td>
<td>Adult 100, Children 20</td>
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<tr>
<td>Pulmonary angiography</td>
<td>Adult 50, Children 10</td>
</tr>
<tr>
<td>Doppler echocardiography</td>
<td>Adult 100, Children 30</td>
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<tr>
<td>Ventilation/perfusion lung scan (V/Q lung scan)</td>
<td>Adult 50, Children 0-10</td>
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<tr>
<td>Lung function tests with DLCO</td>
<td>Adult 100, Children 20</td>
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<tr>
<td>Cardio-pulmonary exercise testing</td>
<td>Adult 20, Children 0-10</td>
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<tr>
<td>6 min walking test</td>
<td>Adult 200, Children 30</td>
</tr>
<tr>
<td>Biomarkers: BNP and troponin</td>
<td>Adult 200, Children 30</td>
</tr>
<tr>
<td>Country</td>
<td>Population (mil)</td>
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<tr>
<td>-------------</td>
<td>------------------</td>
</tr>
<tr>
<td>Poland</td>
<td>37.8</td>
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<tr>
<td>Romania</td>
<td>19.1</td>
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<tr>
<td>Czech Rep.</td>
<td>10.7</td>
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<td>Hungary</td>
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<td>Serbia</td>
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<tr>
<td>Bulgaria</td>
<td>6.9</td>
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<tr>
<td>Croatia</td>
<td>4.1</td>
</tr>
<tr>
<td>Slovenia</td>
<td>2.1</td>
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Therapy for PAH/CTEPH

• PAH
  – three classes of drugs, new coming...
    - all approved based on RCT
    - some Tx expensive/complex (continuous IV/SC administration)
    - upfront double/triple combination - uptitrated, lifelong

• CTEPH
  - surgery – percutaneous angioplasty – and/or drugs
    - complex/costly one-time (surgery) or serial (angioplasty) procedure
    - drugs as pre-treatment and for persistent CTEPH - lifelong
Poland – Access to drug therapy in PAH
Journey from enrollment to first RCTs to reimbursed triple therapy...

12/1998
Enrollment to First RCT in PAH Only monotherapy
- Treprostinil s.c.

1999-2006
More RCT –
- Bosentan,
- Sildenafil,
- Sitaxenton,
- Ambrisentan,
- Iloprost inh.
Individual applications To Ministry of Health for Treprostinil s.c.

2008
Establishing of 1st Drug Program for PAH
Only monotherapy
Products:
- Sildenafil,
- Bosentan
- Iloprost
- Treprostinil

11/2008
Changes in Drug Program:
First combination was approved
Sildenafil + Iloprost

2015
Changes in Drug Program:
Introduction of:
- Veletri i.v. (epoprostenol)
Other combinations
Treatments:
- Macitentan+Sildenafil
- Sildenafil + PC
- Sildenafil + other ERA

11/2018
Changes in Drug Program:
Introduction of:
- Riociguat
- Triple combinations
Sildenafil + Bosentan + PC
Sildenafil + Macitentan + Epo

01/2021
Triple oral Combination therapy
- Selexipag
  + Bosentan/Macitentan + Sildenafil

FC III & IV
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FC II & III & IV
FC II & III & IV
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FC II & III & IV

FC III – sequential combi
FC IV – initial combi

FC II – monotherapy
FC III – sequential combi
FC IV – initial combi

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Access to treatment for PAH/CTEPH - challenges for the future

• Support of **tele-medicine triage** in suspected PH
• Broader access to **CPMS (national and cross-border)** in complex PH cases
• Immediate **access to all validated therapies** for PAH and CTEPH
• In view of progressive pulmonary vascular changes irreversibly affecting QoL and survival **delays in effective Tx are unacceptable**
• Each patient with PAH and suboptimal treatment effects should be discussed with **collaborating lung transplantation team**.
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