



European **M**ulti**P**artner **I**PF **R**Egistry 2nd international SC meeting

Martina Vašáková¹, Martina Šterclová¹, Lenka Mokrá²

1-Thomayer Hospital, Prague, Czech Republic; 2 – Institute of Biostatistics and Analyses, Masaryk University

EMPIRE (European MultiPartner IPF REgistry)

- **International, multicentre, observational, non-interventional registry** of IPF patients in Central and Eastern Europe
- It is independent and opened for multisource support from different subjects
- It is opened to other newly coming partners
- The ownership of data and the access to the data: national pneumologic societies
- **Steering Committee** involves deputies from all participating countries
- Statistical outcomes for purposes of reimbursement (health insurance companies), real-life efficacy of treatment (pharma companies), clinical investigations- epidemiology, prognosis, subgroups comparisons



EMPIRE registry

- Cooperation of Central and Eastern European countries in IPF epidemiology, diagnosis and treatment
- Support of national and European clinical research on IPF-epidemiology, genetics...
- Opportunity to publish data in respected respiratory journals
- Chance for Ph.D. students
- Pool of patients for clinical studies
- Tool for negotiations with healthcare insurance companies and regulatory organs- new treatment modalities in IPF



IPF registry- what are the demands and expectations?

- **High threshold scientific registry-** huge amount of information, including highly specialized investigations, central reading and approval of some data- very demanding on the people involved
 - **What you get-** subcohort of IPF patients from the centers of excellence, specialized on IPF, high scientific value, no real life situation, no chance to have real epidemiologic data
- **Low threshold clinical registry-** less information, no central reading, only consultation on demand
 - **What you get-** real life picture of all patients with IPF who are diagnosed and treated with antifibrotic treatment, almost all of the patients who are not on specific treatment, the results are closer to real epidemiology of IPF and show real life treatment with outcome and prognosis



Collected data

- **Simple to fill in-** only 10 pages CRF in total
- **Inclusion criteria:**
 - Confirmed diagnosis of idiopathic pulmonary fibrosis (IPF)
 - Patient's consent
- **Endpoints**
 - FVC decrease
 - TL_{CO} decrease
 - Death
- **Data-** patients' characteristics and medical history, diagnosis and disease management, functional parameters, HRCT, quality of life (EuroQoL questionnaire)
- **Safety reporting** for the new antifibrotic drugs- also compulsory for reimbursement of new highly innovative drugs
- **Follow-up** of the patients until death- every 3-6 months
- **Data management and statistical analyses** are done by Masaryk University, Institute of Biostatistics and Analyses (IBA), Brno, Czech Republic



Outcome of the registry

Primary outcome:

- To estimate IPF incidence, prevalence and mortality in Central and Eastern Europe

Secondary outcome:

- To describe basic characteristics (e.g. age, gender, risk factors etc)

Tertiary outcome:

- To describe real life approach to IPF in participating countries:
 - diagnostic algorithm
 - treatment patterns and management of patients
 - treatment outcomes (treatment response, overall survival) and quality of life
 - resource utilization
- To determine number of patients suitable for enrolment in clinical trials



Investigator initiated studies from EMPIRE

- Regional (national) and Central-Eastern European epidemiologic data on IPF
- Clinical research- knowledge on patients prognosis, functional parameters and HRCT changes in time
- Compare current treatment with new modalities in real clinical life
- Projects can be proposed by each member- considered by Steering Committee and approved by chair
- Ownership of published data- first author- author of proposal and author of manuscript, co-authors consecutively listed according number of patients enrolled to registry





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(IPF)

Involved countries

Current status of EMPIRE – involved countries

- **Czech Republic**
 - 10 sites already participating in IPF registry in the Czech Republic
- **Hungary (5 sites)**
- **Poland (6 sites)**
- **Slovakia (6 sites)**
- **Serbia (1 site)**
- ***Turkey, Israel* – in progress**





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**eCRF changes since last SC
meeting**



Current User: Access EN Test (TEST_IPF_EN)

Current project: EMPIRE

Time to log out: 59:53

Log Out

Search Patient

Add New Patient

Patient's Forms

Patient

Tools

Data form End of observation

Patient

IPF-XX01-008

Date of birth (dd.mm.yyyy)	01.01.1950	Initials	ab	Enrolled by	Access EN Test
Sex	Male	Site	CBA2	Date of enrollment	22.04.2015

Data form

Save

Save and close

End of follow-up

End of follow-up *

Diagnosis changed

Patient status at the time of end of follow up

Lung transplantation

Date of lung transplantation (dd.mm.yyyy) *

Death

Date of death (dd.mm.yyyy) *

Cause of death

Patient lost to follow-up or other reason

Date of last contact (dd.mm.yyyy) *

Please specify another reason for the end of observation

Diagnosis changed

Date of diagnosis reconsideration (dd.mm.yyyy) *

Specification *

eCRF changes since last SC meeting

- Validation criteria

Data form Follow-up

Patient

IPF-XX01-008

Date of birth (dd.mm.yyyy)	01.01.1950	Initials	ab	Enrolled by	Access EN Test
Sex	Male	Site	CBA2	Date of enrollment	22.04.2015

Data form


Save

Save and close

Recalculate

Follow-up


The interval between follow-ups should be at least 3 months. If patient died please fill in the form End of Observation (do not fill follow-up any more with information that the examination was not performed because of patient's death).

Date of visit (dd.mm.yyyy) * 


Control visit performed *

Reason for not performing the control visit

Weight (kg) *

03.09.2015 

No 

Patient did not arrived for the visit 



Adverse Event

- Based on final
- From novel drugs where holder was
- Reduced

sponsor (BI) we
t form
related to
horization
partment

Search Patient Add New Patient Patient's Forms Patient Tools

Data form Adverse Event

Patient

IPF-XX01-008

Date of birth (dd.mm.yyyy)	01.01.1980	Initials	ab	Enrolled by	Access ERI Test
Sex	Male	Site	CSA2	Date of enrollment	22.04.2015

Data form

Save Save and close Recalculate

Basic information

Type of report

Weight

Height

Pregnancy

Weeks

Type of adverse event

Type of the adverse event	Please specify	Grade
<input type="text"/>	<input type="text"/>	<input type="text"/>

+ Add record -X Delete record

Adverse Event

Onset date of the adverse event (dd.mm.yyyy) *

Stop date of the adverse event (dd.mm.yyyy)

Was the adverse event serious (SAE)? *

Specify serious adverse event

Death ☐

Life-threatening event ☐

Patient required hospitalization or existing hospitalization was prolonged ☐

Persistent or significant disability/incapacity ☐

Consequential anomaly/birth defect ☐

Required intervention to prevent permanent impairment or damage ☐

Is there a reasonable causal relationship with the drug administered? *

What is the indication of this drug? *

Type of non-IPF indication

IPF Medication

Please specify

Who is the marketing authorization holder?

Specify

Is the patient treated with this drug within the clinical trial (CT)?

Is the patient treated with this drug within the compassionate use program (CUP)?

Was the Adverse Event already reported within the CUP or CT?

Please insert CUP/CT number and sponsor

Please insert site number in the CT/CUP

Please insert patient ID in the CT/CUP

Assessment of the adverse event

AE treatment *

Type of treatment

Formulation

Total daily dose at onset (dose, unit)

Route of administration

Start date of treatment of AE (dd.mm.yyyy)

End date of treatment of AE (dd.mm.yyyy)

Indication for use

Concomitant therapy (relevant)

Is there a reasonable causal relationship between the event and the concomitant therapy?

Concomitant diagnoses (relevant)

Outcome of AE

Action taken with suspect drug due to event

Comments

Please insert telephone number and email in case any further details are needed





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(IPF)

Frequently asked question(s)

FAQ

- **Where to enter FVC information?**
 - FVC data are part of the Follow-up form only
 - Discrepancy: Protocol Data collection time points (p.10)

MONTH/DATE	DATA COLLECTED
0m	Entry form, Treatment
Every 3m/6m	Treatment, Follow-up Information , Quality of life form, Hospitalisation, Adverse Event (if AE occurred)
End of observation	End of observation

? Protocol adjustment ?

- Man: $(FVC) \cdot 100 / ((27.63 - (0.112 \cdot \text{age at the time of diagnosis})) \cdot \text{height on the Enrollment form} / 1000)$
- Woman: $(FVC) \cdot 100 / ((21.78 - (0.101 \cdot \text{age at the time of diagnosis})) \cdot \text{height on the Enrollment form} / 1000)$



Predicted value

- Man: $(FVC) * 100 / ((27.63 - (0.112 * \text{age at the time of diagnosis})) * \text{height on the Enrollment form} / 1000)$
- Woman: $(FVC) * 100 / ((21.78 - (0.101 * \text{age at the time of diagnosis})) * \text{height on the Enrollment form} / 1000)$





EMPIRE
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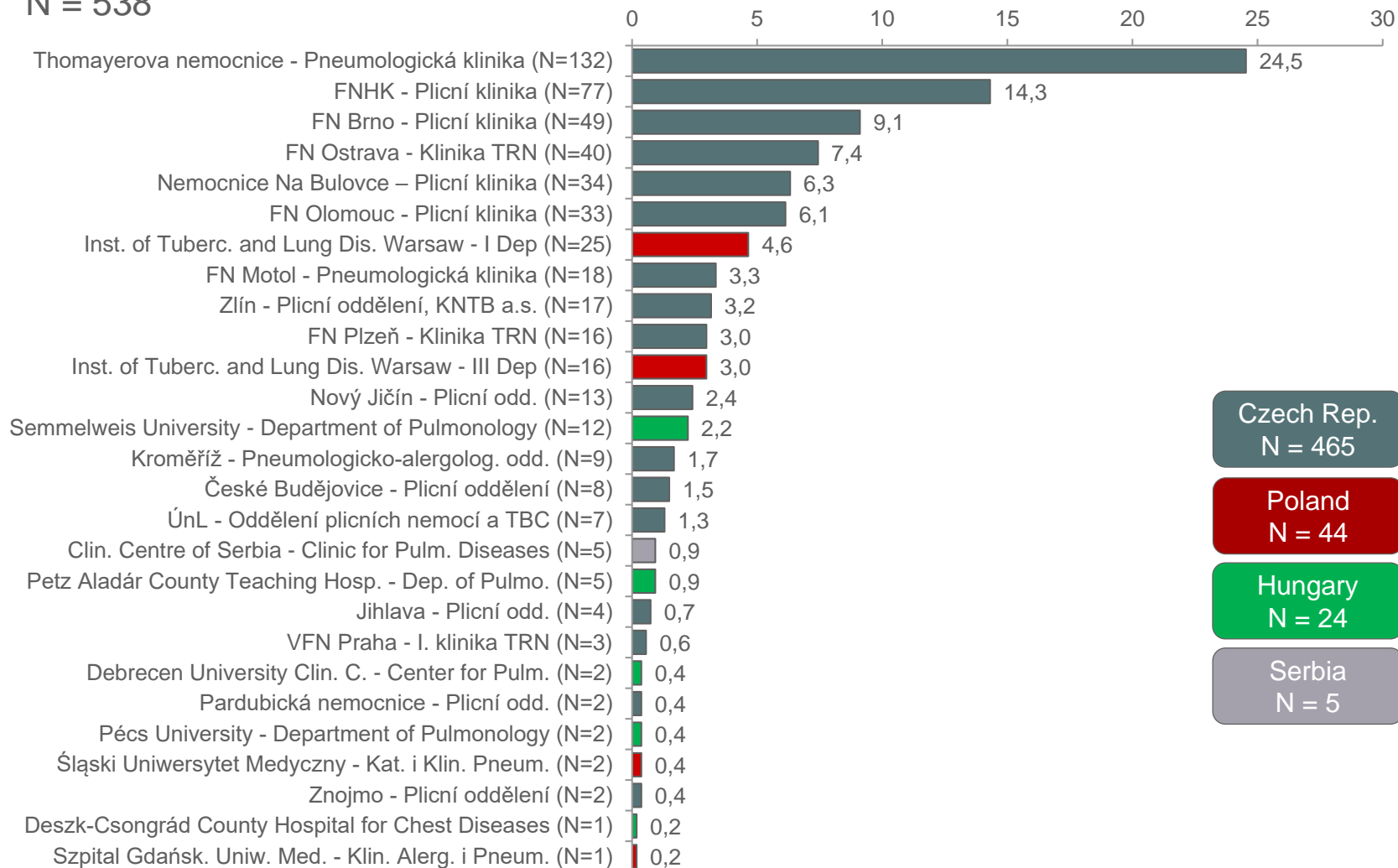
Data analysis

Data export 31AUG2015

Number of patients in EMPIRE registry

N = 538

% of patients



Czech Rep.
N = 465

Poland
N = 44

Hungary
N = 24

Serbia
N = 5



Pharmacological treatment

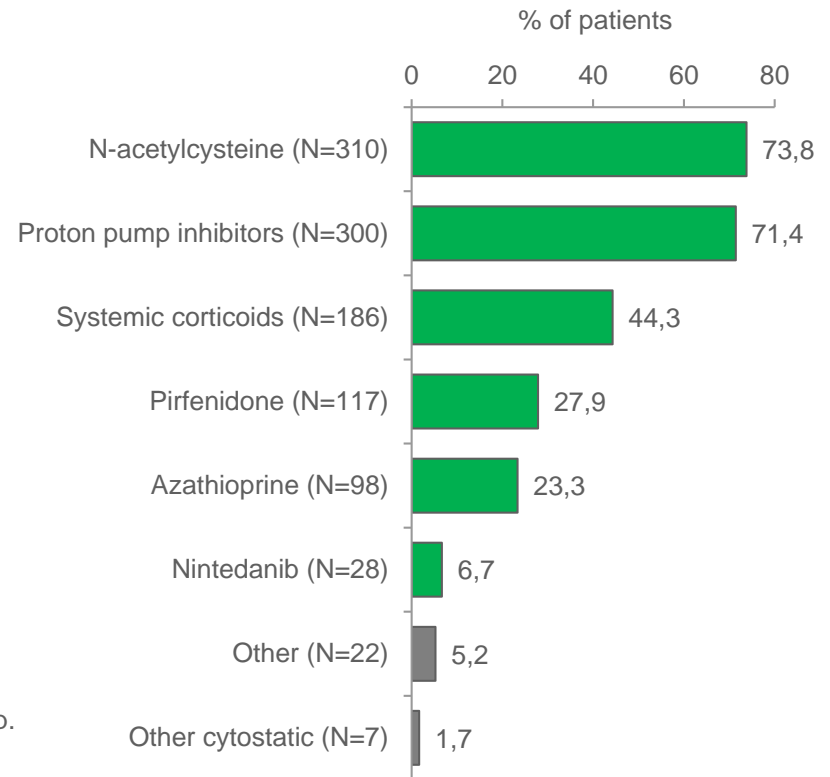
N = 538

Type of treatment (known N)	N (%)
Pharmacological treatment (N=503)	420 (83.5%)
Rehabilitation (N=501)	203 (40.5%)
Oxygen therapy (N=503)	145 (28.8%)
Lung transplantation (N=501)	79 (15.8%)
Clinical Study (N=502)	39 (7.8%)

1 patient could have more types of treatments during follow up.

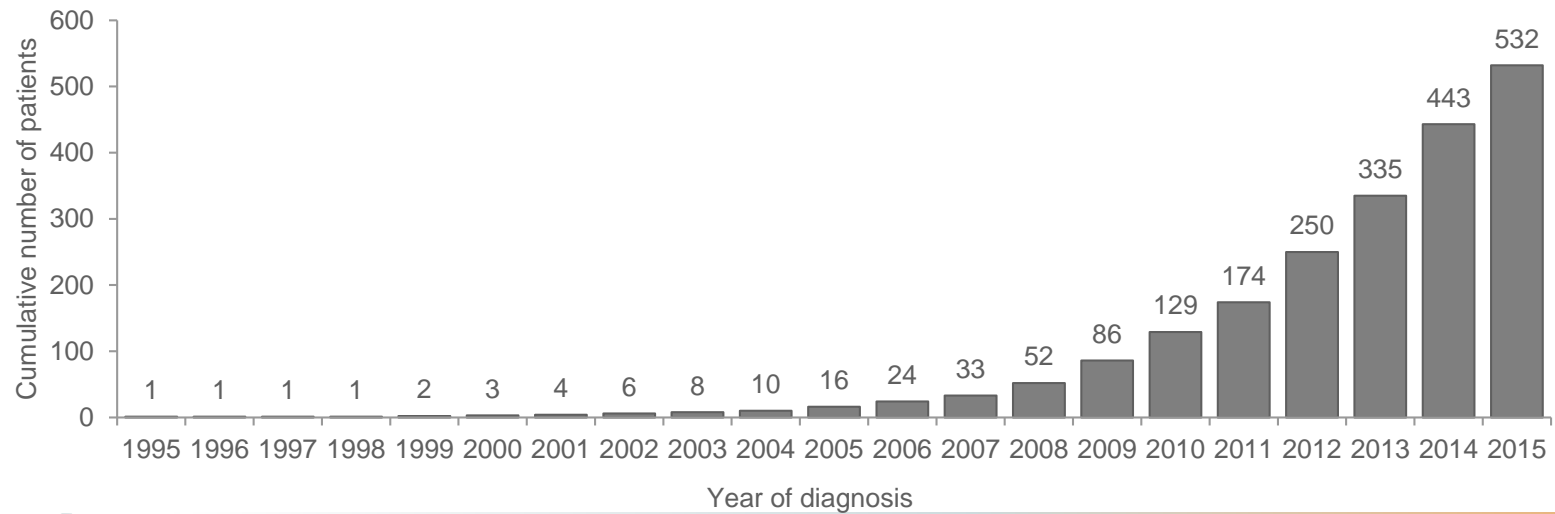
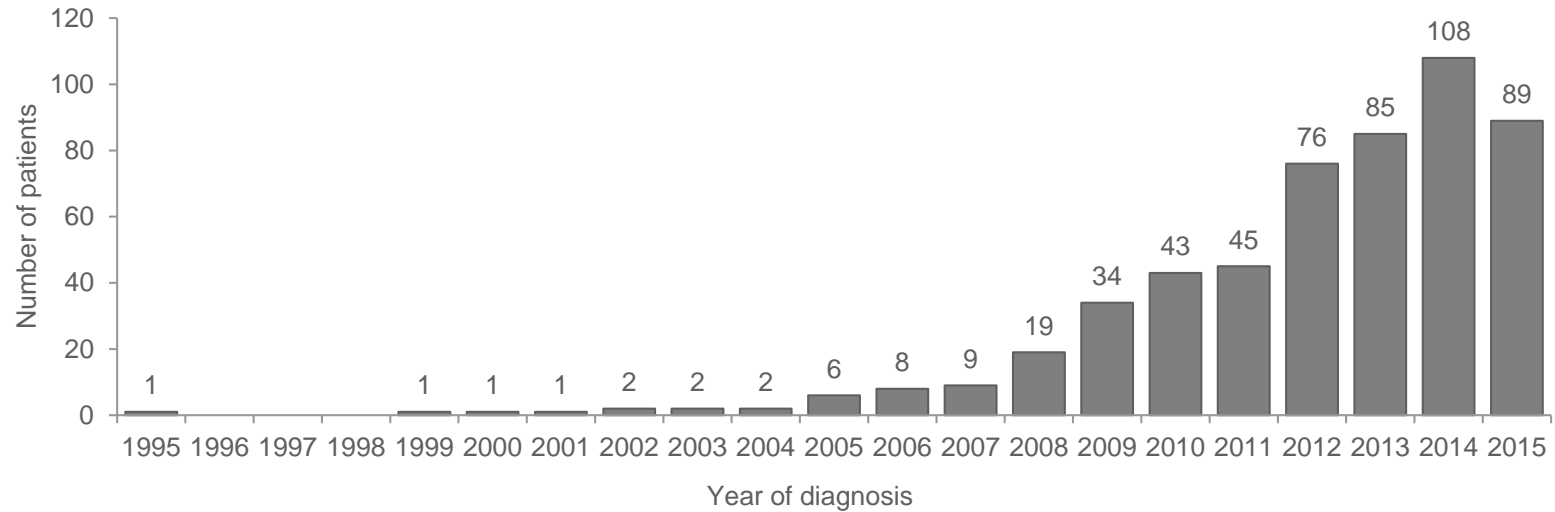
1 patient with pharmacological treatment could use more drugs during follow up.

N = 420



Time of diagnosis

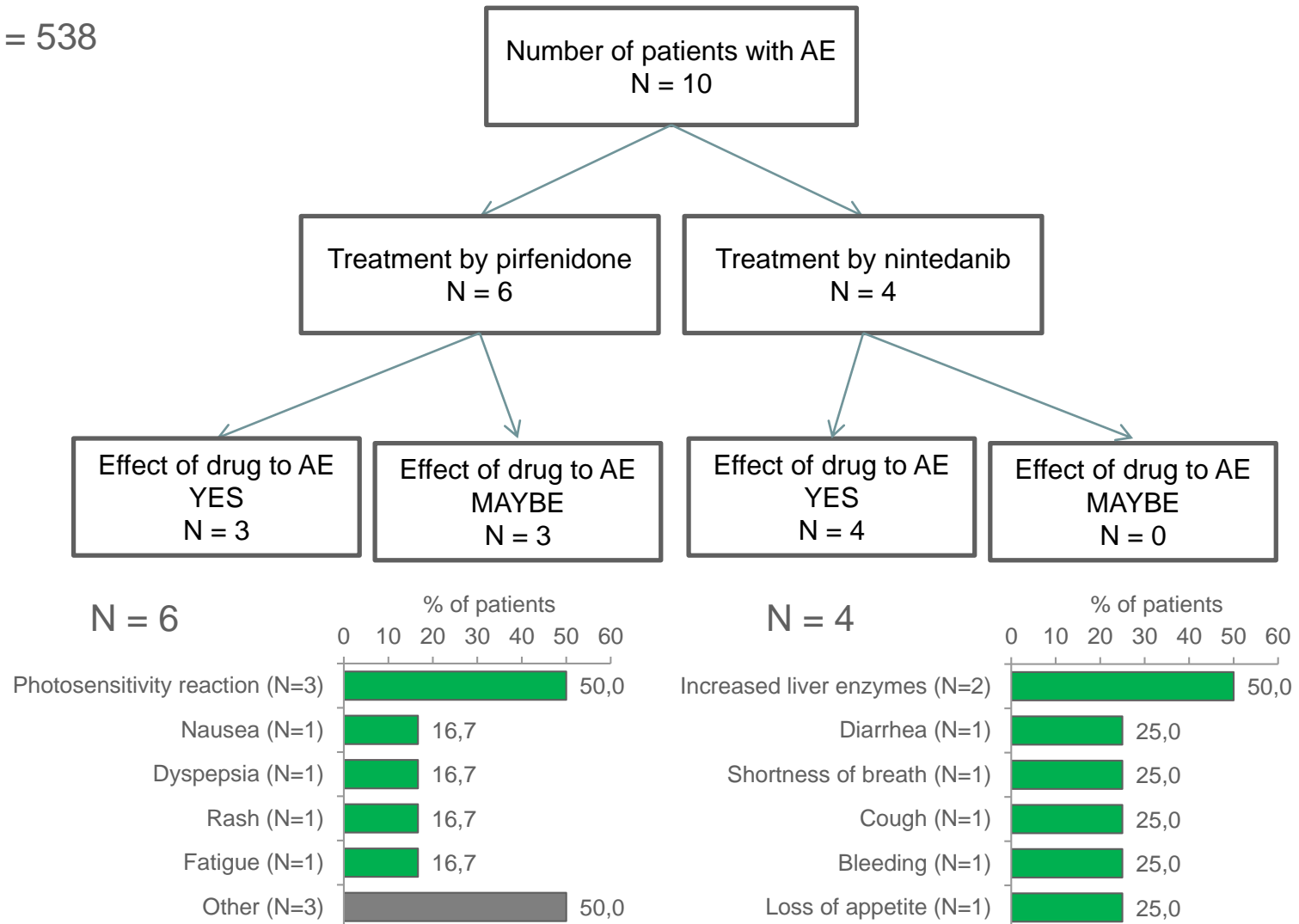
N = 538



No information about date of diagnosis in 6 patients.

Adverse events (AE)

N = 538



No information about type of AE in 1 patient with nintedanib





EMPIRE
(IPF)

Current and further projects on EMPIRE data

The projects currently running in EMPIRE registry

- Early diagnosis of IPF- does it influence survival?
- Phenotypes of IPF (only the Czech part)
- Genetics of IPF (only the Czech part)
- Others???



To be discussed

- Opinion with web site and protocol
- Protocol change?
- Independency should be emphasized-
multisource financial support is invited
- In IPF registry should be optimally all cases
of IPF, not only the treated ones
- This ppt available on EMPIRE website?





EMPIRE
(IPF)

**Thank you for attendance on the
2nd international SC meeting**