

# Personalized medicine in Chronic Kidney Disease

PICKED and DISCO-I Joint Meeting,  
May 13–15, 2025 | Toulouse, France

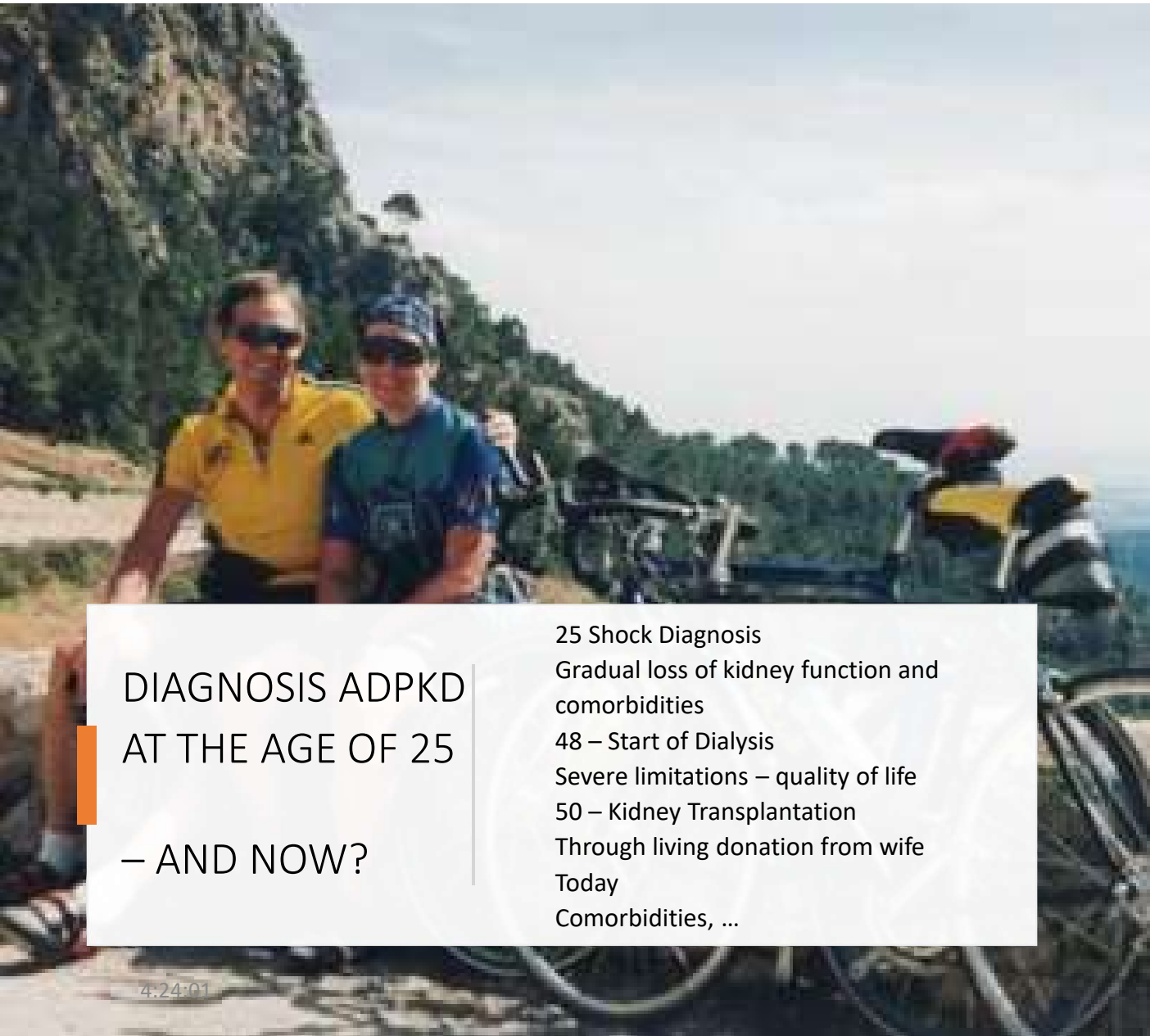
**Patient Views:** Uwe K.H. Korst

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- PICKET (=Patient Inclusion in Chronic Kidney Disease Education and Decision-making)
- DISCO (Decision-making in Serious and Chronic Conditions)



**Kidney diseases are silent  
killers impacting 100 million  
Europeans**



## DIAGNOSIS ADPKD AT THE AGE OF 25

– AND NOW?

25 Shock Diagnosis  
Gradual loss of kidney function and  
comorbidities  
48 – Start of Dialysis  
Severe limitations – quality of life  
50 – Kidney Transplantation  
Through living donation from wife  
Today  
Comorbidities, ...

4:24:01



*"The diagnosis hit me like a shock: a shortened life expectancy, being advised against having children, and the prospect of early dialysis. Even today, there is a lack of effective treatments and adequate care for people with rare diseases. Urgent improvements in healthcare are essential."*



**What do patients need?  
Summary**

# General requirements for patients

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Enhance shared decision-making between patients and healthcare providers. (Sorry: But not all have communications skills)



Provide education and resources tailored to patients' needs, empowering them to make informed decisions about their care.



Promote patient involvement in research and healthcare policy to ensure their perspectives are included in decision-making processes.

# Patient needs (1)

## 1. Mental & Emotional Well-being

### 1) Physical health

(pain, fatigue, mobility, daily activities)

**Treatment burden** (Side effects, time spend on therapies, insurance issues)

**2. Emotional Well-being** (anxiety, depression, mental resilience), Psychological support

### 3. Social life

(relationships, family, social acitivities)

Independence

### 4. Financial / work impact

Access to healthcare, treatment costs, ability to work

## 2. Early Detection

Kidney disease is a silent killer

Holistic Approach: Rare kidney diseases (e.g., PKD) affect **multiple** organ systems, not just the kidneys.

Genetic testing, better markers

Biomarkers (Cre isn't a good marker)

Registries

## 3. Medical care and treatments

**Access to specialists** (e.g., nephrologists, geneticists for rare diseases)

**Personalized treatment plans** tailored to their needs

**Availability of innovative therapies & clinical trials**

**Regular monitoring & early intervention**

**European-wide registries**

###For what #####

Slow the progression: Risk Management: Non-Medical Impacts: Effects on quality of life, work, family, social participation, and mental health.

Shared decision-making and patient involvement

Education and resources

## 4. More research needed

Develop (new) therapies based on individual (genetic) profiles. Research explores

Personalized medicine

Research on (new) drugs for kidney diseases

Slow down progression with targeted treatments

Use of AI

innovative intervention strategies to improve patient outcomes

European-wide registries

## 5. Economic burden → political support

(European) National kidney plans for:

Early Detektion

Expert Centers

Support of Research

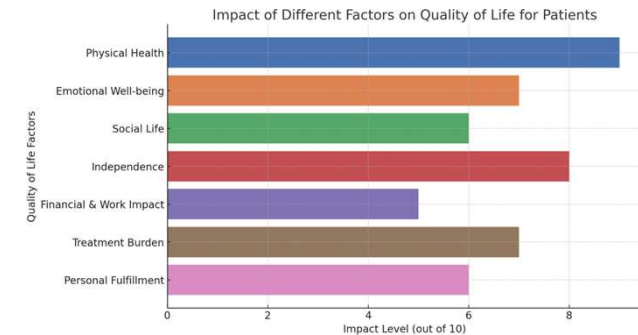
Support of registries

European initiatives

.....

To 1. Patients needs  
Mental & Emotional Well  
being (QoL)

# Quality of life (QoL) Mental & Emotional Well being



**Overall well-being**, encompassing physical, psychological, and social aspects. It goes beyond medical outcomes and looks at how the disease and its treatment impact daily living. Key factors influencing QoL for patients include:

**1. Physical Health:** Pain levels, fatigue, mobility, and ability to perform daily activities.

**Treatment Burden:** Side effects, complexity of medical regimens, and time spent on therapies.

→ medicine and care givers

**2. Emotional Well-being:** Anxiety, depression, and mental resilience in dealing with the disease.

→ Psychological support (therapy, counseling, or patient groups)

→ Empowerment through education (understanding the disease & self management)

**3. Social Life:** Ability to maintain relationships, participate in family life, and engage in social activities.

→ Supportive family & social environment

**4. Financial and Work Impact:** Ability to continue working, financial burden of treatment, and access to healthcare.

For chronic and rare disease patients, QoL is often reduced by long-term symptoms, uncertainty about the future, and limitations in social participation. Patient-centered care aims to improve QoL by addressing these factors, not just treating the disease itself.

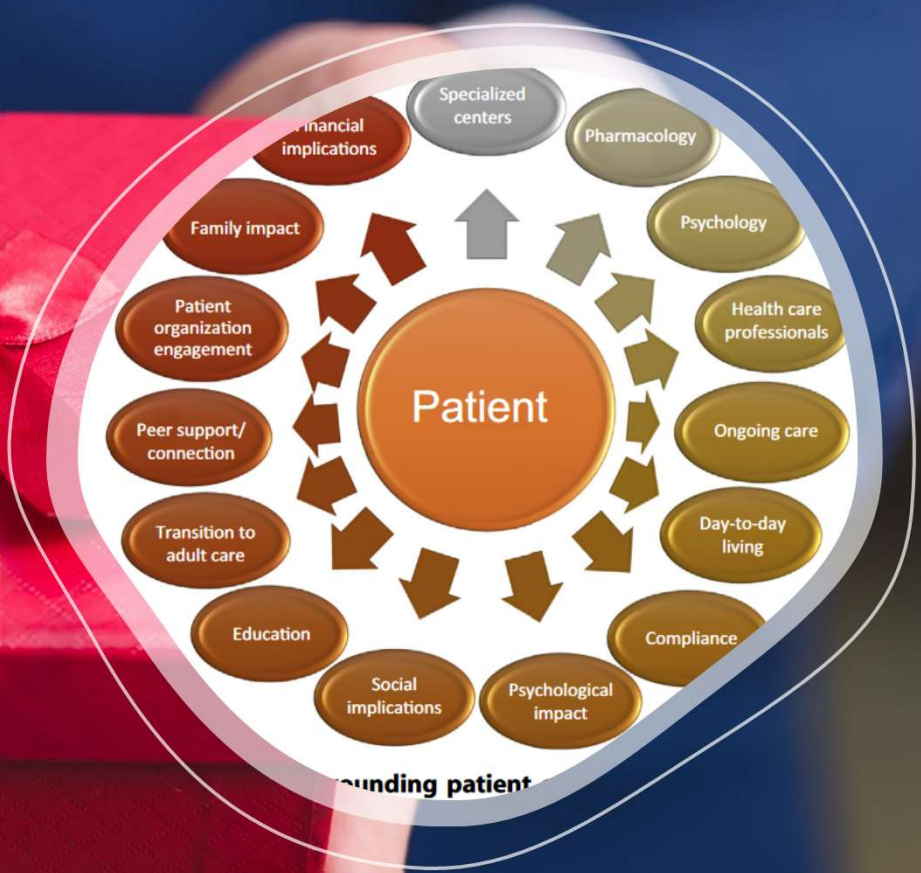


## Key questions of patients after diagnosis

- 1. Disease Status:** “How serious is it? Will it progress quickly?” Will I need dialysis? What are my options? Understanding the disease.
- 2. Treatment:** “Which (individual) treatments or medications do I need now and in the future?” What are side effects?
- 3. Lifestyle Changes:** “Do I need a special diet, fluid restrictions, or exercise plan?”
- 4. Monitoring:** “How often do I see my doctor and which tests do I need?”
- 5. Complications:** “What risks (like high blood pressure and other organs) should I watch for?”
- 6. Dialysis or Transplant:** “Will I need dialysis or a transplant and how (long) do those work?”
- 7. Finances:** “Will insurance cover my treatments? Are there assistance programs?”
- 8. Emotional Support:** “Where can I get counseling and/or mental health help or join a support group?”
- 9. Quality of Life:** “Can I keep working, traveling, or doing my usual activities?”

Goal:  
Maintain quality of life  
as much as possible

KDIGO-view  
Improving Global Outcomes (KDIGO)  
Controversies Conference



Supporting patient

**To 2. Patients needs –  
Early detection**

# Patient Journey through diagnosis

“It’s a waiting game, but you tell a mum to wait when she’s waited 15 years. It’s difficult. – Nuria

“People began to ask which side of the family it came from...It was a difficult time for us as parents. – Alexa

“A diagnosis may be bad news, it may be very bad news or it may be no news. But all of that’s OK and there’s help and support for whatever spectrum you end up on. – Peter

“We went around, travelling across the entire city to find a nursery for our son. It was impossible to have him accepted. – Gaston



Information and training for patient organizations | Helplines | Information on secondary findings | Next Steps Toolkit | EUROGENTEST Guidelines | Experience Based Co-Design | RareConnect.org | ENSERIO study: Time to Diagnosis | Undiagnosed Photo Project | Protocol to support ultra-rare diagnosis | Training for professionals

<https://www.eurordis.org/publications/solve-rd-infographic-on-the-patient-journey-to-diagnosis/>

# Why is early detection so important?



**Risk:** The low incidence of the disease poses a risk for patients of a delayed, missed, or incorrect diagnosis and a delayed referral to expert centers.



Early diagnosis is essential to prevent irreversible kidney (and other organs) damage.

The cost of diagnosing must be weighed against the cost of missing it.



**Patient consensus  
summit, Brussels  
26-27 January**

**Patient leaders and  
globally leading kidney  
organizations**

**Recommendations  
on how to improve  
kidney health**

# Kidney health in Europe, Action plan

**Implement broader screening programs** to secure early detection of common and rare kidney diseases.

**Secure broad and timely access to quality care** through equitable access to experts, medication and opportunity to participate in relevant clinical trials for CKD.

**Support transplantation** through strengthened donation programs.

**Prioritize kidney health in healthcare strategies and plans**, to the benefits of patients, their families and society.

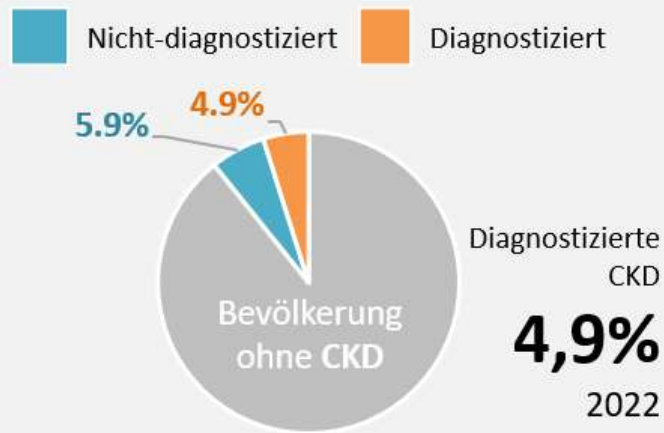
**Promote awareness of CKD**, incl. its impact on the quality of life, and its financial- and environmental consequences.



*The meeting was funded by the German Selbshilfegruppe for Komplementerkrankungen, Novartis, Sobi, AstraZeneca & Alexion*

# LESS THAN HALF OF CKD PATIENTS ONLY ARE DIAGNOSED (DATE FROM GERMANY)

## CKD-Prävalenz Stadium 1-5 + unbekannt



Diagnoseraten sind niedrig

**45,2%** Diagnostiziert **vs** **54,8%** Nicht-diagnostiziert

Anzahl der Hospitalisierungen – CKD Patient:innen

**4,48 Mio** 2018 **-13%** **3,89 Mio** 2022

Anzahl aller Hospitalisierungen in DE<sup>a</sup>

**19,81 Mio** 2018 **-13%** **17,21 Mio** 2022

Hospitalisierungsrate relativ zur CKD-Kohortengröße

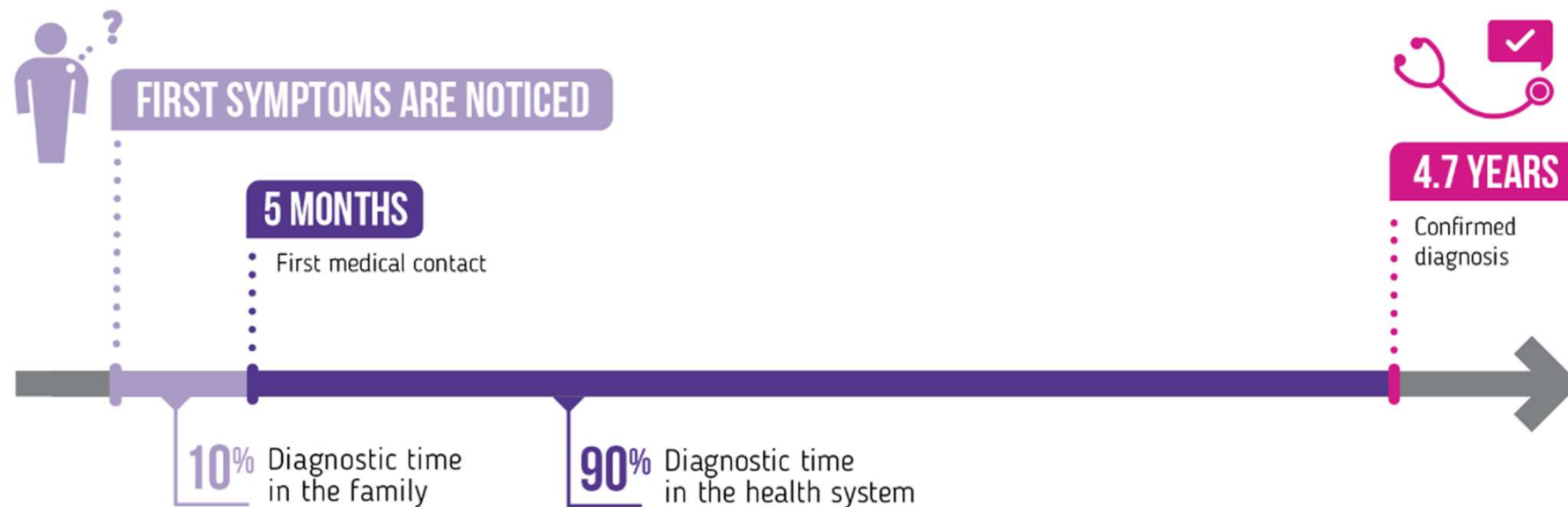
**1,13** 2018 **-16%** **0,95** 2022

a) Global, regional, and national burden of chronic kidney disease, 1990–2017: a systematic analysis for the Global Burden of Disease Study 2017, Lancet, 2020

a) Statistisches Bundesamt: Eckdaten der Krankenhauspatient:innen; <https://www.destatis.de/DE/Themen/Gesellschaft-Umwelt/Gesundheit/Krankenhaeuser/Tabellen/entlassene-patienten-eckdaten.html> sowie [https://www.statistischebibliothek.de/mir/receive/DEHeft\\_mods\\_00131401](https://www.statistischebibliothek.de/mir/receive/DEHeft_mods_00131401)

## 2. THE DIAGNOSIS ODYSSEY: A LONG JOURNEY

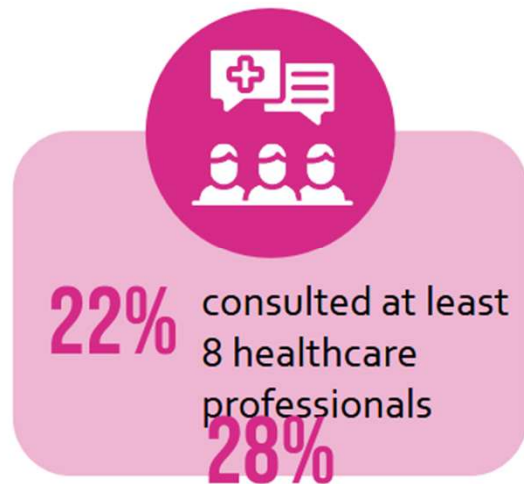
Most of the diagnostic time stems from health systems



[https://download2.eurordis.org/rarebarometer/Webinar\\_RB\\_Diagnosis\\_Results\\_30\\_01\\_2025.pdf](https://download2.eurordis.org/rarebarometer/Webinar_RB_Diagnosis_Results_30_01_2025.pdf)

# 3. THE DIAGNOSIS ODYSSEY: A DIFFICULT JOURNEY

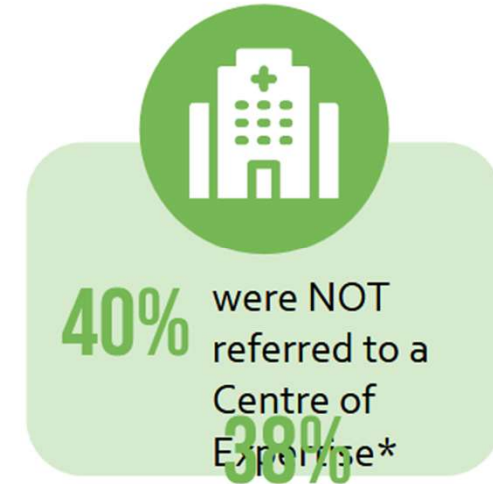
## Associated difficulties



of those diagnosed within one year consulted at least 5 healthcare professionals



of those diagnosed within one year were misdiagnosed at least one time



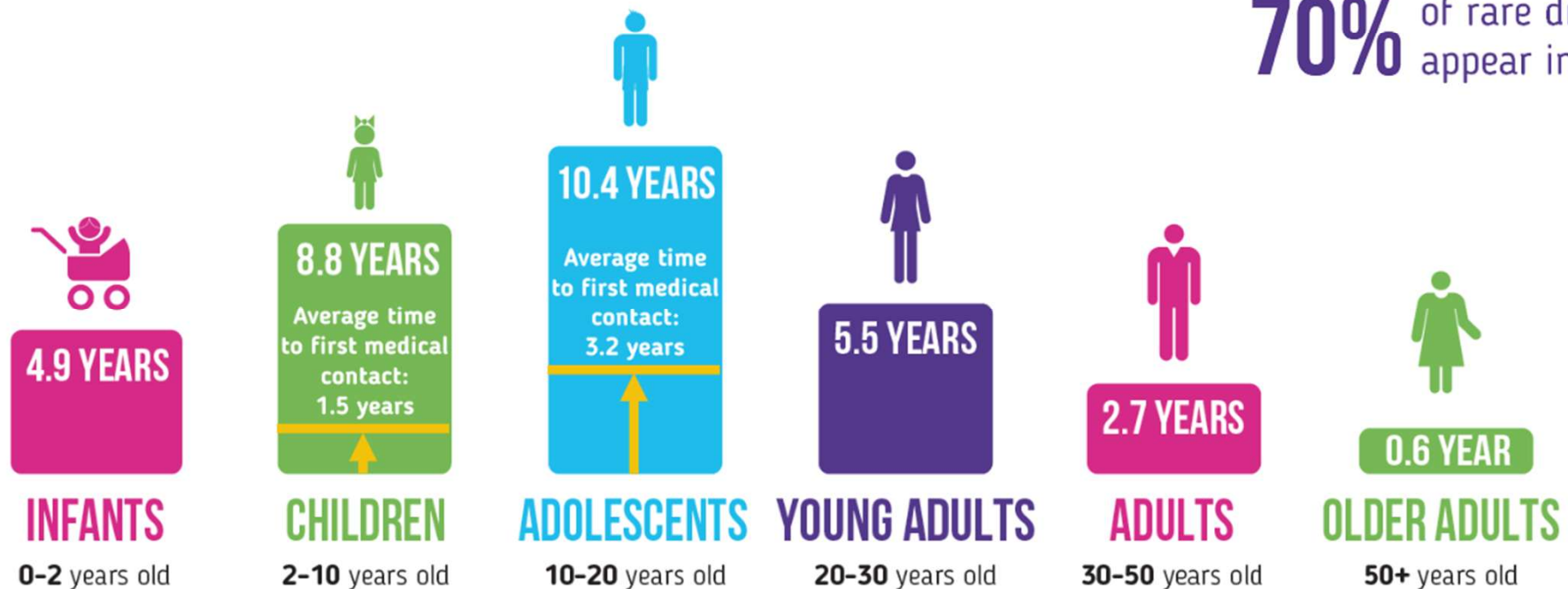
of those diagnosed within one year were NOT referred to a Centre of Expertise

\*A Centre of Expertise is a hospital unit specialised in a rare disease or in a group of rare diseases  
[https://download2.eurordis.org/rarebarometer/Webinar\\_RB\\_Diagnosis\\_Results\\_30\\_01\\_2025.pdf](https://download2.eurordis.org/rarebarometer/Webinar_RB_Diagnosis_Results_30_01_2025.pdf)

# 5. MAIN DETERMINANTS OF DIAGNOSTIC DELAYS

## Age at first symptoms

**70%** of rare diseases appear in childhood



Average number of years between first symptoms and confirmed diagnosis depending on the age of the patient at symptom onset  
Logit: OR = 2.26 for infants; 3.10 for children; 4.74 for adolescents; 2.44 for young adults; 1,70 for adults; ref=older adults.

# Importance of early diagnosis



# To 3. Medical care and treatment

(guidelines should address  
personalization)

### 3. Medical care and treatment

**(Europeanwide-) Access to specialists** (e.g., nephrologists, geneticists for rare diseases)

**Personalized treatment plans** tailored to their needs

**Availability of innovative therapies & clinical trials**

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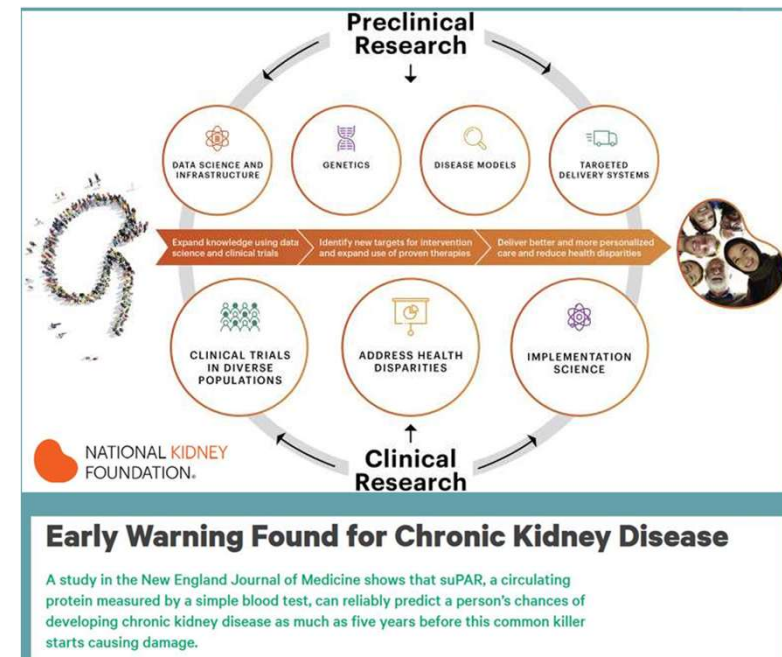
**European-wide registries**

## 4. More research needed

(European registries, guidelines should address personalization)

# More research needed

- Develop (new) therapies based on individual (genetic) profiles.  
Research explores -- Personalized medicine
- (Bio-) markers for early detection
- Re-Use of drugs
- Research on (new) drugs for kidney diseases
- Slow down progression with targeted treatments
- Use of AI
- Innovative intervention strategies to improve patient outcomes
- European-wide registries



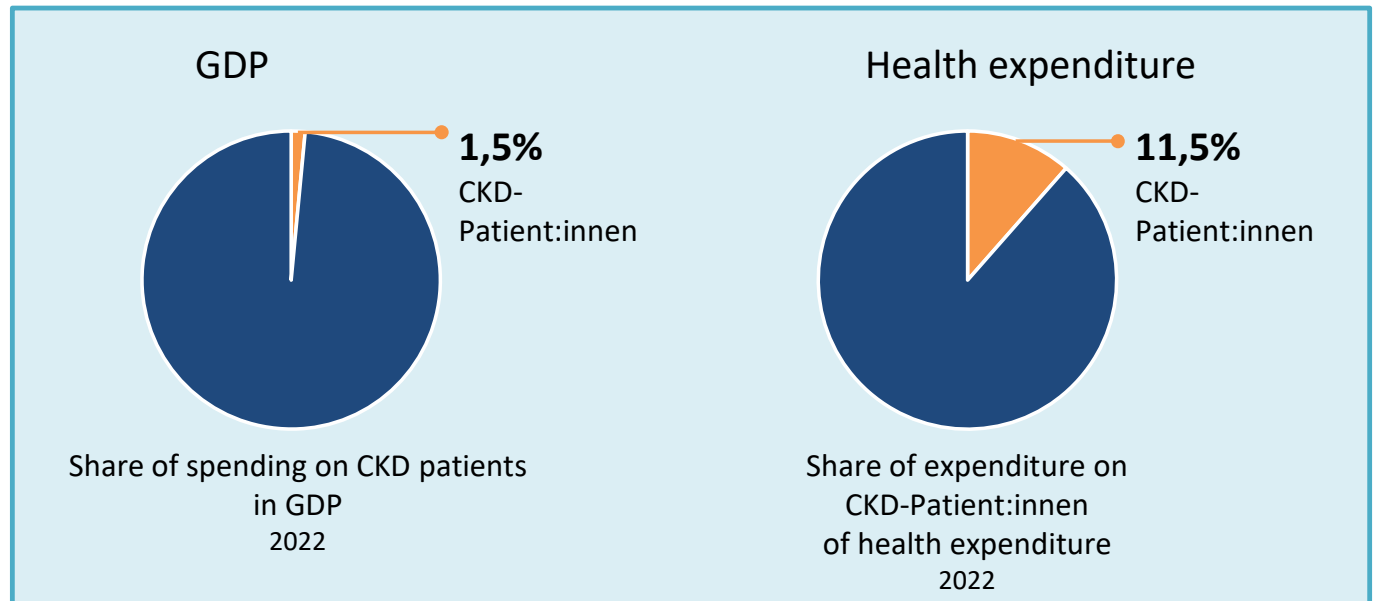


# 5. Economic burden of society

# MORE THAN 10% OF TOTAL HEALTHCARE SPENDING IN GERMANY IS SPENT ON PATIENTS WITH CKD

Gross inland product  
(GDP)<sup>a</sup>  
**€3,870 billion**  
2022

Health expenditure EN<sup>b</sup>  
**€498 billion**  
2022



Diagnosed CKD patients, 4.9% of the total population, account for 11.5% of total healthcare spending. Due to the low diagnosis rate, the costs may be underestimated!

a) Federal Statistical Office, <https://www.destatis.de/DE/Themen/Wirtschaft/Volkswirtschaftliche-Gesamtrechnungen-Inlandsprodukt/Publikationen/Downloads-Inlandsprodukt/inlandsprodukt-erste-ergebnisse-pdf-2180110.html>

b) Federal Statistical Office, [https://www.destatis.de/DE/Themen/Gesellschaft-Umwelt/Gesundheit/Gesundheitsausgaben/\\_inhalt.html#235030](https://www.destatis.de/DE/Themen/Gesellschaft-Umwelt/Gesundheit/Gesundheitsausgaben/_inhalt.html#235030)

# Society needs

Other aspects:

- European wide registries, knowlege exchange,
- Economical aspects safes money
  - 10 % of healthcare costs
  - ? % of of GdB due to loss of work hours





## Summary: Cost of treatment

- The further the kidney disease progress without diagnosis and treatment, the greater the financial burden for both patients, caregivers and healthcare systems.
- The costs of long-term management, including dialysis, potential transplantation, medicine, etc. in addition to the loss of productivity of the affected individual and potentially also the caregivers, is substantial.

Improved healthcare  
and (individual)  
effective treatments  
are absolutely  
essential!

- *"The diagnosis hit me like a shock: a shortened life expectancy, being advised against having children, and the prospect of early dialysis. Even today, there is a lack of effective treatments and adequate care for people with rare diseases. Urgent improvements in healthcare are essential."*

- Thank you for listening