A Prospective, Observational, Basket, Comprehensive Cohort Study of Children and Adolescents Aged Less Than 18 Years with Anemia Associated with Chronic Kidney Disease Requiring or Not Requiring Dialysis.

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Primary:Evaluate the impact of SoC anemia management on Hgb concentrationsSecondary:-Characterize the use of ESA therapies in management of anemia associated with moderate to severe CKD in children and adolescents aged

Ethical review	Approved WMO
Status	Will not start
Health condition type	Nephropathies
Study type	Observational invasive

Summary

ID

NL-OMON53656

Source
ToetsingOnline

Brief title
CoCoA-CKD
Comprehensive Cohort of Anemia in Chronic Kidney Disease

Condition

- Nephropathies

Synonym
Anemia, Chronic Kidney Disease

Research involving
Human

Sponsors and support

Primary sponsor: GSK Research & Development Limited
Source(s) of monetary or material Support: GlaxoSmithKline Research & Development Limited

Intervention

Keyword: Anemia, Chronic Kidney Disease, CKD, Observational

Outcome measures

Primary outcome

At each study time point:

• Hgb value.

• Hgb change from baseline (Day 1).

• Hgb above, below and within the range 10 to 12 g/dL.

During the study:

• For participants who initiate ESA therapy for the first time during the

study, the Hgb value at the time of ESA initiation, and 4 and 8 weeks after ESA

initiation.

Secondary outcome

ESA usage at each study time point described as:

• ESA usage overall and by type

• ESA dose and standardized ESA dose

• ESA dose changes from baseline (Day 1)

During the study:

• Incidence of ESA initiation

• Incidence of all AEs and Serious Adverse Events

(SAEs).

• In addition, although there is no study intervention, the incidence of:

o Adverse Events of Special Interest (AESI) defined in this protocol.

o AEs leading to discontinuation of ESA SoC therapy.

• Changes from baseline in laboratory safety parameters, blood pressure (BP),

heart rate (HR), height and weight at each time point.

Study description

Background summary

Anemia is common in children with chronic kidney disease (CKD), affecting up to 58% of patients with stage 2 CKD and 93% of patients with stage 5 CKD. Anemia can be treated by correction of iron deficiency, treatment with erythropoietin stimulating agents (ESAs) or blood transfusion, but all of these have important limitations. Furthermore, considerable uncertainty exists in the classification of, and treatment targets for, anemia of CKD in a pediatric population.
Research priorities include the following:
• Understanding the current, real-world management of anemia in children and adolescents with CKD.
• Understanding how hemoglobin (Hgb) concentration responds to current standard-of-care (SoC) practices.
• Evaluating novel agents for the treatment of anemia in this setting.

This observational Cohort Study aims to address all of these research priorities by establishing a cohort of pediatric patients with anemia associated with CKD and:
1. Capturing a detailed anemia and anemia treatment history at study entry (historical medical records review).
2. Evaluating Hgb response and other key health outcomes to SoC interventions (prospective observation).
3. Serving as a population of informed, research-active patients with defined clinical need, from which to offer accelerated enrollment into the planned (GlaxoSmithKline [GSK] Study 214066 with daprodustat for renal anemia) or potential future nested interventional or detailed observational studies.

Study objective

Primary:
Evaluate the impact of SoC anemia management on Hgb concentrations

Secondary:
- Characterize the use of ESA therapies in management of anemia associated with moderate to severe CKD in children and adolescents aged <18 years
- Evaluate the incidence of adverse events (AEs) and other safety parameters

Study design

This is a prospective, international, multicenter, observational cohort of approximately 234 pediatric participants who have treated or untreated anemia associated with CKD not requiring dialysis (ND subpopulation) or dialysis-dependent (D subpopulation).

Informed consent will also include for a retrospective review of a participant*s medical records for up to the 12 months preceding study entry, as well as consent to approach for the interventional trial 214066, when they become eligible for approach

Study burden and risks

This study takes up to 3 years depending on whether or not additional 3-monthly visits are required. It is expected that most participants will be finished with their participation in this study after 1 year and will have received an invitation to the interventional study 214066 in which a new drug called Daprodustat is investigated.

During this study, participants will visit the hospital every 3 months. The assessments are similar to those done during standard of care visits. If recent blood sampling results are available, blood draws might not need to be done. The following test, procedures and assessments can be done during a visit in this study:

- Ask you questions about your demographics
- Ask you questions about your ethnic background. We do this to see if different groups of people might respond differently to their treatments.
- Ask you questions about your medical and treatment history. We will also ask you questions about your kidney transplant and/or dialysis status if applicable.
- Ask questions about how you are feeling, which medications you are taking and any side effects you are experiencing.
- Physical Examination
- Assessment of your physical development to see the effect of your disease on your growth.
- Assessment of your physical development
- Measure your height and weight

- Measure your vital signs, e.g. blood pressure and heart rate.
- Assessment of your physical development to see the effect of your disease on your growth.
- Blood sampling if required.

During each visit you might also receive an invitation to the other study in which a new medicinal product is tested. This depends if your age group has opened and how well your kidneys function. All participants will receive an invitation.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Children (2-11 years)
Babies and toddlers (28 days-23 months)
Newborns

Inclusion criteria

1. Participants must be <18 years of age at the time of signing of informed consent.
Note: Infants born prematurely (under 32 weeks of gestation) should have a chronological age of at least 6 months.

2. Participants who have anemia associated with CKD as follows:
ND subpopulation: CKD stage 3, 4, 5 (not on dialysis) based on eGFR using the bedside Schwartz equation <60 mL/min/1.73 m2
D subpopulation: CKD Stage 5d
AND
Receiving treatment for anemia with an ESA
OR
In ESA non-users, a Hgb <=12.0 g

3. The investigator, or a person designated by the investigator, will obtain written informed consent from each study participant's (legal guardian as defined in section 10.1.3) and the participant's assent, when applicable, before any study-specific activity is performed (unless a waiver of informed consent has been granted by an IRB/IEC). All legal guardians should be fully informed, and participants should be informed to the fullest extent possible, about the study in language and terms they are able to understand.

4. The participant capable of providing signed and dated written assent signs and dates a written assent form (age-appropriate) and the parent/guardian signs and dates a written ICF for study participation prior to the initiation of any study-related activities. The informed consent is described in Section 10.1.3.

5. A legal guardian or primary caregiver must be available to help the study site personnel ensure follow-up; supporting the participant to attended assessment days according to the SoA.

Exclusion criteria

1. Kidney transplant recipient with a functioning allograft.
2. Scheduled for elective kidney transplantation within 3 months.
3. Aplasias: History of bone marrow aplasia or pure red cell aplasia.
4. Other causes of anemia: e.g., thalassemia major, sickle cell disease or myelodysplastic syndrome.
Note: Sickle cell trait is acceptable if the participant otherwise meets entry criteria.
5. Unresolved acute or active chronic infection requiring antimicrobial therapy.
6. History of malignancy within the prior 2 years or currently receiving

treatment for cancer or renal lesions, for which, in the opinion of the investigator, malignancy cannot be excluded.
Note: In the case of localized squamous cell or basal cell carcinoma of the skin that has been definitively treated, the exclusion timeline is within the last 4 weeks.
7. Cirrhosis or current unstable liver or biliary disease per investigator assessment defined by the presence of ascites, encephalopathy, coagulopathy, hypoalbuminemia, esophageal or gastric varices or persistent jaundice.
Note: Stable non-cirrhotic chronic liver disease (including Gilbert's syndrome, asymptomatic gallstones and chronic hepatitis B or C) are acceptable if the participant otherwise meets entry criteria.
8. Participants who are currently participating in a clinical study with a study intervention (IMP or licensed drug), which is under evaluation to treat anemia associated with CKD or is anticipated to confound the interpretation of the Hgb status of the participant.
9. Participants with any history of hypersensitivity to daprodustat or its excipients, or to any other HIF-PHI.
10. Any other condition, clinical or laboratory abnormality, or examination finding that the investigator considers would put the subject at unacceptable risk, which may affect study compliance or prevent understanding of the aims or investigational procedures or possible consequences of the study.
11. Participants who are receiving a HIF-PHI (including daprodustat), whether licensed or within a clinical trial.

Study design

Design

Study type: Observational invasive

Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Other

Recruitment

NL

Recruitment status:	Will not start
Enrollment:	12
Type:	Anticipated

Ethics review

Approved WMO Date:	31-01-2023
Application type:	First submission
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	19-06-2023
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
CCMO	NL80168.091.22