Zorgconsumptie, Economische Analyse, Levenskwaliteit, Overleving, Uitkomsten, en Scores van patiënten met Acute Myeloïde Leukemie

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Primary Objective:- To assess and describe Quality of Life throughout a patients treatment trajectorySecondary Objective(s):- To assess and describe how frail unselected real world AML patients are at diagnosis using geriatric screening tools (G8, 6...

Ethische beoordeling	Afgewezen
Status	Zal niet starten
Type aandoening	Leukemieën
Onderzoekstype	Observationeel onderzoek, zonder invasieve metingen

Samenvatting

ID

NL-OMON51315

Bron
ToetsingOnline

Verkorte titel
ZEALOUS-AML

Aandoening

- Leukemieën

Synoniemen aandoening
AML, bloedkanker, leukemie

Betreft onderzoek met
Mensen

Ondersteuning

Primaire sponsor: Medisch Centrum Leeuwarden
Overige ondersteuning: Medisch Centrum Leeuwarden

Onderzoeksproduct en/of interventie

Trefwoord: AML, Farmacoeconomie, Kwetsbaarheid, Levenskwaliteit

Uitkomstmaten

Primaire uitkomstmaten

• Quality of Life reported by patient

• Overall survival

Secundaire uitkomstmaten

• Rate of Complete Remission or Complete Remission with incomplete count recovery CR/CRi

• Early Death (30-day mortality)

• Ferrara Criteria treatment advice

• Wheatley Score

• HCT-CI Score

• AML-CM Score

• ECOG Performance Status

• Cytogenetica / molecular risk profile

• ELN classification

• Other parameters necessary for scoring such as eGFR, LDH, RBC, BMI, Albumine, Serum Creatinine

• Treatment regimen as per 2 weeks after diagnosis

• Healthcare usage such as antibiotic prescription events or receiving blood transfusion

• Quality of Life as reported posthumous by relatives

A complete list of datapoints collected and how they will be formatted in our

research database can be found in the excel file *Databasestructure

ZEALOUS-AML*.

Toelichting onderzoek

Achtergrond van het onderzoek

Acute myeloid leukemia (AML) is a malignant disease of the bone marrow with a poor prognosis, especially in elderly patients [1][2]. With a year incidence of 3 - 4,5 cases per 100.000 people AML is a rare disease although it is the most frequently diagnosed leukemic disease [3]. As the median age at diagnosis is 68 years, AML at a young age is viewed as a different disease entity [1][2][3]. Patients aged below 65 are commonly treated by intensive induction chemotherapy until remission or remission with minimal residual disease is reached. Remission, if reached, is then consolidated with an allogenic or autologic stem cell transplant. Elderly patients are often times not in a good enough baseline health state to undergo this highly intensive, somewhat risky, and overall expensive treatment trajectory [1][3]. In 2012 and 2015 respectively the Hypomethylating agents Azacitidine and Decitabine were introduced to the market and offer less intensive treatment options for elderly patients [4][5]. Because of this, stratifying de novo AML patients into the follow three groups became common practice: Fit for intensive chemotherapy, not fit for intensive chemotherapy but fit enough for HMA based therapy, and unfit even for HMA based therapy [6]. After a period in which Azacitidine monotherapy was the first choice of treatment for patients deemed ineligible for intensive induction therapy, the combination of Azacitidine AZA with Venetoclax VEN was proven to lead to superior outcomes and has been promoted to first choice of treatment for this patient group in March 2022 [7]. In the near future, multiple other agents currently in phase III clinical trials are expected to make it into treatment guidelines [8][9].
The superiority in terms of OS of the HMA with VEN combination regimens over HMA comes with more side effects, adverse events, nights stayed in the hospital (time toxicity), as well as increasing healthcare costs. It is unknown how Quality of Life (QOL) develops in AML patients under the novel regimens in the real world setting [10]. It would be highly desirable to be able to predict which patients develop toxicity to better identify patients that might benefit from regimens such as AZA+VEN There are multiple prognostic tools for evaluation of certain conditions and interventions already validated in AML populations (Ferrara criteria, Wheatley Index, HCT-CI, AML-CM, and more being developed) [6][11][12]. It is unknown however how valuable these tools are for aiding clinical decision-making. It is also not well known what other costs

-besides the substantial cost of agents such as Venetoclax- the new regimens creates through increases in healthcare usage.

Doel van het onderzoek

Primary Objective:
- To assess and describe Quality of Life throughout a patients treatment trajectory

Secondary Objective(s):
- To assess and describe how frail unselected real world AML patients are at diagnosis using geriatric screening tools (G8, 6-CIT) and comorbidity tools (HCT-CI, AML-CM, Ferrara criteria lists, Wheatley Index, Charlson Comorbidity Index CCI)
- To determine of what predictive quality of clinical scoring tools (Ferrara, Wheatley, HCT-CI, AML-CM) are for endpoints such as overall survival, 1-year survival, remission rates, rates of treatment related mortality, and early death (within 30 days)
- To evaluate the potential of the Wheatley Index, the HCT-CI, and the AML-CM for usage as a tool to support choosing between non-intensive chemotherapy and best supportive care in AML
- To analyze how an AML patients QOL changes in respect to time and in respect to treatment regimens
- To quantify healthcare usage (transfusions, antibiotics, other) of elderly patients with AML.
- To evaluate the AZA+VEN regimen from a pharmaco-economical perspective (ICER, price per QALY) by combining the survival-, quality of life-, and healthcare usage data and comparing it to existing data on Azacitidine monotherapy

Onderzoeksopzet

The ZEALOUS-AML study is a prospective, observational, single-centre, real world study. The investigators are exploring options for collaboration with other STZ hospitals. The inclusion period of the study is 2,5 years (between 01-01-2023 and 31-06-2025). The design was chosen for its real world representativeness, aiming for inclusion of at least 50 patients in the Medisch Centrum Leeuwarden. Assessment of comorbidity scores requires access to a patients full medical history. Quantification of healthcare usage and pharmacoeconomic evaluation require extensive data gathering during a patients trajectory. All datapoints collected are required for the analysis. An interim pharmacoeconomic analysis may commence as early as when the 15th included patient passed away.

Inschatting van belasting en risico

The study is mainly observational in nature with questionnaires for patients

(before cycle 1, after cycle 1, after cycle 2, after cycle 4, 6, 8, *) and family (2-4 weeks after death of the patient) being the only interventions. AML patients spend considerable time in the hospital anyways, especially during the first cycle. We deem the risk for patients to be zero and the burden for patients to be acceptable with each questionnaire taking approximately 30 minutes of their time. The burden for the family is potentially significant as they are approached with an optional request in a difficult time of their life, that however only takes 5-15 minutes to answer.

Contactpersonen

Publiek

Medisch Centrum Leeuwarden

Henri Dunantweg 2
Leeuwarden 8934AD
NL

Wetenschappelijk

Medisch Centrum Leeuwarden

Henri Dunantweg 2
Leeuwarden 8934AD
NL

Locaties

Landen waar het onderzoek wordt uitgevoerd

Netherlands

Deelname eisen

Leeftijd
65 jaar en ouder

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

Patients in the Dutch province of Friesland may present at any of the 4 Frisian Hospitals. Complicated cases such as elderly patients with AML are referred to the MCL. Patients from outside of the province of Friesland are uncommon.

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

A potential subject who meets any of the following criteria will be excluded from participation in this study:
• Age upon diagnosis <= 65 years
• Patients diagnosed with APL (t(15;17), WHO 2016)

Onderzoeksopzet

Opzet

Type: Observationeel onderzoek, zonder invasieve metingen

Blindering:	Open / niet geblindeerd
Controle:	Geen controle groep
Doel:	Organisatorisch/zorgonderzoek

Deelname

Nederland

Status:	Zal niet starten
Aantal proefpersonen:	100
Type:	Verwachte startdatum

Ethische beoordeling

Afgewezen

Datum:	03-04-2023
Soort:	Eerste indiening

Toetsingscommissie:	RTPO, Regionale Toetsingscie Patientgebonden Onderzoek (Leeuwarden)

Registraties

Opgevolgd door onderstaande (mogelijk meer actuele) registratie

Geen registraties gevonden.

Andere (mogelijk minder actuele) registraties in dit register

Geen registraties gevonden.

In overige registers

Register	ID
CCMO	NL83339.099.22