CBmed Strategic Project

HTA-type early assessment of biomarkers

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Introduction

As part of the CBmed Health Technology Assessment research programme, the Institute HEALTH – Institute for Biomedicines and Health Sciences (JOANNEUM RESEARCH) undertook two partial Health Technology Assesment (HTA)-type assessments of biomarkers for (i) minimal residual disease (MRD) detection in acute myeloid leukaemia (AML) and (ii) pulmonary hypertension (PH). The EUnetHTA Core Model® was used as a methodological basis for the assessments.

Aims

- Identify the relevant patient groups for the two biomarkers in question and estimate the number of patients for whom biomarker testing would be relevant
- Identify the current care situations and treatment pathways for these patients
- Estimate the potential impact of the new biomarkers on care and treatment pathways
- Identify potential costs and where possible potential cost savings areas that could be attributable to these biomarkers, in relation to the current situation

Methods

Systematic evidence analysis of clinical guidelines and clinical and epidemiological studies, together with survey of expert opinion and compilation of cost data for Austria, as detailed in our previous CBmed poster (Schmidt et al, 2016).

Results

- **AML:** The assessment suggests that MRD assessment in patients with acute myeloid leukemia at certain stages of the disease and therapy could be a valuable tool, in particular for adjusting the risk status stratification of patients in the post-induction phase (see figure 1). As well as improving outcomes through better tailored treatment, potential savings to the Austrian health care system of € 4.5 million could result from the more appropriate use of consolidation chemotherapy and transplantation.
- **PH:** Three potential clinical indications for use of the PH biomarker were identified: (1) diagnosis of pulmonary hypertension as a cause of unexplained chronic dyspnea (1-4% of patients presenting in primary care); (2) monitoring of patients receiving PAH therapy (11,000-44,500 patients in Europe) and (3) identifying COPD patients with concomitant PAH who might benefit from PAH therapy (around 150,000 patients per year in Europe).

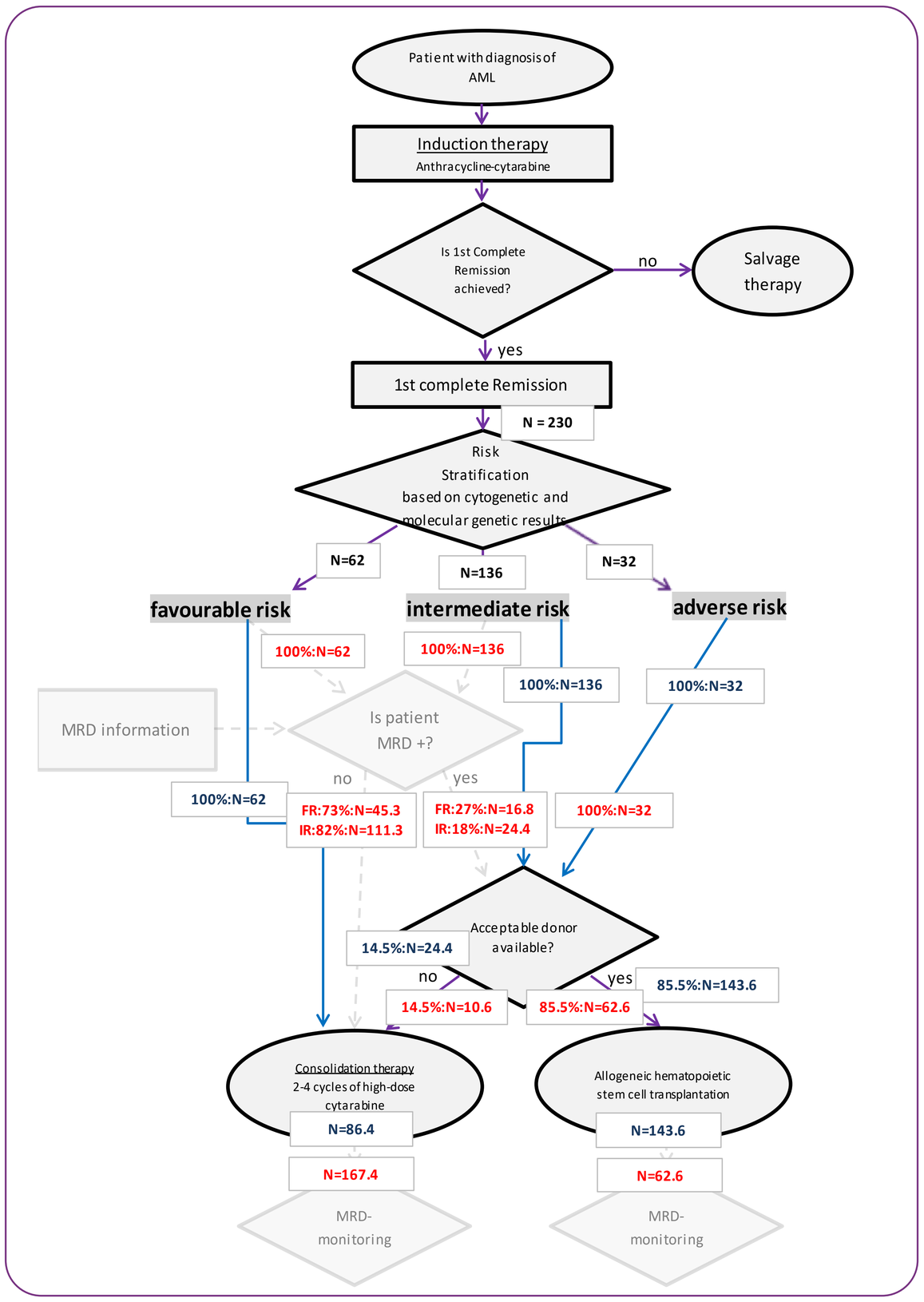

Figure 1: Quantification of current (numbers in blue) and future (numbers in red) based on MRD clinical practice for the Austrian setting. (FR: favourable risk. IR: intermediate risk).

Conclusions

- **AML:** Potential clinical use and patient relevance could be clearly identified and demonstrated. In addition to savings in health care costs, societal savings in terms of productivity gains are possible for working age patients.
- **PH:** The present analysis shows that a biomarker for pulmonary hypertension has the most potential for use as a companion diagnostic tool for monitoring expensive PAH therapy.

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