Background: Randomized controlled trials (RCTs), which are currently considered to provide the highest level of evidence, include patients with high disease activity and exclude those with comorbidities often seen in the real world. With the increasing recognition of the importance of real-world evidence, attention is being paid to discrepancies between RCT-based evidence and the patient population in routine clinical practice; however, few reports assessing these gaps in the context of rheumatoid arthritis (RA) are available.

Objectives: We investigated the discrepancy between the efficacy of biological DMARDs (bDMARDs) in routine clinical practice in patients with RA based on the IORRA logical DMARDs (bDMARDs) in RCTs and the effectiveness of bDMARDs in routine clinical practice in patients with RA based on the IORRA cohort.

Methods: The effectiveness of bDMARDs (etanercept [ETN; n=23], golimumab [GLM; n=20], certolizumab [CZP; n=17], abatacept [ABT-subcutaneous injection (sc); n=14], and tocilizumab [TCZ-sc; n=24]) in RA patients who newly initiated bDMARD therapy in our hospital in 2016 was compared with the efficacy reported in phase 2 or 3 trials (8 total RCTs: ETN, 1; GLM, 2; CZP, 2; ABT, 1; TCZ, 2) during the RCT’s observatioinal periods. Effectiveness was evaluated by percentages of patients achieving ACR20, ACR50, and ACR70 and clinical remission based on DAS28 remission criteria using the IORRA cohort database. We also compared treatment responses between IORRA eligible and non-eligible patients in the MATSURI study, a phase 2 trial that evaluated TCZ-sc efficacy and safety and had relatively lenient inclusion criteria, since some RA patients in the IORRA cohort belonged to both the eligible and non-eligible groups.

Results: In 7 RCTs (excepting the MATSURI study), very few patients fulfilled the IORRA inclusion criteria (Table). The ACR achievement rates were higher among patients in RCTs, while remission rates were higher among IORRA patients (Table). The average DAS28 at baseline in the RCTs group was significantly higher than that in the IORRA group (Table). In the MATSURI study, 13 of 24 patients who newly initiated TCZ-sc met the inclusion criteria in the IORRA. Although the percentages of patients achieving ACR20, ACR50, and ACR70 and clinical remission among eligible patients were similar to those of patients in the MATSURI study, those of non-eligible patients were lower than those of MATSURI patients (Table). This indicates better effectiveness in eligible patients than in non-eligible patients.

Conclusion: Although RA patients in RCTs experienced a better ACR achievement rate than those in routine practice, clinical remission was difficult to achieve. This might be due to the difference in disease activity at the time of bDMARD introduction, suggesting that it is necessary to interpret the efficacy of RCTs based on differences in patient background.

REFERENCES

Table
