

the assessment (2). Ten of them were implemented in stage 3 for congestive heart failure and pulmonary obstructive chronic disease. Significant values were found both in ABS with and without chronicity care programmes (phase 3).

### CONCLUSIONS:

The subsequent analysis (phase 4) will allow identification of practices of each ABS that best explain these results. Some limitations must be considered such as the availability of the consensued indicators in the SISCAT databases.

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2. Grup de Treball d'Avaluació de la Cronicitat. Desenvolupament d'un marc conceptual i indicadors per avaluar l'atenció a la cronicitat. Segon informe. Barcelona: Agència de Qualitat i Avaluació Sanitàries de Catalunya. Departament de Salut. Generalitat de Catalunya; 2013.

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## VP175 Validating Outcome Assessments For Health Technology Assessment In Ceroid Lipofuscinosis Neuronal 2 (CLN2), An Ultra-Rare Disease

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### INTRODUCTION:

Ceroid lipofuscinosis neuronal 2 (CLN2) disease, a form of Batten disease, is a rare, degenerative neurometabolic disorder. Disease onset around 2–4 years is followed by

rapid decline in motor and neurologic function and mortality in early teenage years (1). Disease burden is best captured using observer-reported outcomes. However, validation is challenging in ultra-orphan diseases, requiring flexible methods and reasonable acceptance of limitations related to participant access.

The study aim was to assess content validation of clinical trial measures (i) CLN2 Disease Based Quality of Life Assessment (Sponsor-developed), (ii) EQ-5D-5L, (iii) Pediatric Quality of Life Inventory (PedsQL); and (iv) PedsQL Family Impact Module.

### METHODS:

The Batten Disease Family Association recruited United Kingdom caregivers of a child with CLN2 disease (aged 3–7 years, non-participants in any CLN2 trial), to:

1. Focus groups with symptom elicitation
2. Cognitive interviews to assess measures.

### RESULTS:

The Focus group comprised eleven caregivers (eight female, three male) from six families. Three families were current caregivers and remainders bereaved. Symptom and disease impact elicited showed the majority of measures domains were relevant.

The interview sample comprised sixteen current caregivers (twelve female, four male) from ten families (caring for eleven children). Overall measures were relevant, easy to understand and answer. However several items were difficult to apply to children with advanced disease (for example, Euroqol, EQ-5D-5L “overall health”), when ability is lost (for example, PedsQL walking), with misinterpretation of “no difficulties” with eating where child feeds using gastrostomy (CLN2 QoL). Caregivers found it difficult to know how their uncommunicative child was feeling (PedsQL worrying, EQ-5D-5L depression). Some symptoms and impacts were missing (for example, constipation, working life).

## CONCLUSIONS:

The mixed-methods approach enabled content validity assessment of multiple measures. While these measures were largely relevant, adjustments could strengthen these for use in this fatal pediatric condition population and increase their acceptance within health technology assessment (HTA).

## REFERENCES:

1. Mole S E, Williams RE. 2001. Neuronal Ceroid-Lipofuscinoses. In: Pagon RA, Adam MP, Ardinger HH, Wallace SE, Amemiya A, Bean LJH, Bird TD, Fong CT, Mefford HC, Smith RJH, Stephens K, editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993–2016. 2001 Oct 10 [updated 2013 Aug 1]

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## VP176 Effectiveness Of Anti-Tumor Necrosis Factor In Patients With Psoriatic Arthritis

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### INTRODUCTION:

Anti-tumor necrosis factor drugs (anti-TNF) are the last line of treatment for psoriatic arthritis (PsA) in the guideline of Brazilian Public Health System (SUS). Data of effectiveness of these drugs are scarce in the Latin American population. This study evaluated the effectiveness of the anti-TNF on a cohort of patients with PA in the SUS.

### METHODS:

PsA patients treated with anti-TNF, were included in an open prospective cohort study. The Bath Ankylosing Spondylitis Disease Activity Index (BASDAI) and Clinical Disease Activity Index (CDAI) were used to assess the effectiveness at six months of follow-up. The anti-TNF

was considered effective when the patient achieves scores of four or less measured for BASDAI or scores of ten or less for CDAI. Frequency distributions were compiled for the sociodemographic variables and mean and standard deviation (SD) was used for clinical variables. The paired Student t-test was established to evaluate the differences between baseline and 6 months evaluated for BASDAI and CDAI.

### RESULTS:

Fifty-four patients with PsA completed six months of follow-up. The mean age of patients was 54.03 years (10.44) and the mean disease duration was 8.00 years (7.49). Furthermore, 50 percent of the patients were female, 61.1 percent white and 59.6 percent married. The most used anti-TNF was adalimumab (63.0 percent), followed by etanercept (20.4 percent) and infliximab (16.7 percent). The anti-TNF reduced disease activity measured by BASDAI and CDAI at six months of follow-up ( $p < .001$ ). The percentage of patients achieving the effectiveness with anti-TNF was 61.1 percent measured by BASDAI and 53.7 percent by CDAI.

### CONCLUSIONS:

Anti-TNF drugs demonstrated to be effective in more than half of patients at six months. This result highlighted the importance of the treatment with the anti-TNF drugs in the Brazilian population. Long-term data are needed to confirm these results.

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## VP177 Older People With Cancer: To Treat Or Not To Treat With Chemotherapy?

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### INTRODUCTION:

Older people with cancer are less likely to receive radical treatment for cancer. We conducted a series of systematic reviews to explore the effectiveness and