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| **Supplementary Table 1:** Disease and product profile template **Objective: To present the incremental clinical benefit, incremental budget impact, level of evidence and level of innovation** | | | |
| **Core area** | **Topic** | **Sub-topic** | **Rationale for consideration / objective** |
| **Disease background** | Epidemiology | Prevalence | Impact on size of target patient population Impact on cost profile over time |
| Incidence |
| Patient population characterization | Age of onset | Impact on willingness to pay Impact on clinical uncertainties for specific subgroups |
| Existence of patient subpopulations |
| Other patient characteristics (e.g. gender, ethnicity, risk factors, genetic mutation) |
| Disease manifestation | Disease progression (e.g. acute vs. chronic, attacks) | Characterize the disease profile, which sets up for the treatment goals  Identify the urgency to treat |
| Impact on mortality and life expectancy / prognosis |
| Impact on morbidity |
| Impact on quality of life |
| Extent of variability in disease presentation and severity |
| Economic burden of disease | Economic and societal impact (e.g. missed school days, productivity) | Present a holistic view of the disease impact; this influences the perception of burden of disease sets up for discussion of spillover effects |
| Diagnostic pathway | Time to diagnosis | Impact on start/stop rules, reimbursement criteria (e.g. restriction to sub-populations), budget impact |
| Diagnosis method (e.g. biomarker test, clinical differential, diagnosis of exclusion) |
| Diagnosis rate |
| Other elements |
| Patient care pathway | Availability of clinical guidelines | Qualify how the patient care experience is structured in the health system; this helps identify potential changes to the health system brought about by the novel treatment |
| Patient experience |
| Treatment capacity in the healthcare system |
| Treatment location (e.g. center of excellence) |
| Standard of care | Existence of (a) therapeutic option(s) (approved or off-label) | Quantify the extent of unmet medical need |
| Efficacy of SoC |
| Safety of SoC |
| Cost of SoC |
| Date of introduction of SoC |
| Shortcomings of SoC |
| **Product background** | Technology presentation | Treatment goal (e.g., curative, disease modifying, symptom management, slow progression, attack prevention ) | Impact on perception of innovativeness  Impact on complexity of administration  Impact on cost structure and variability in costs |
| Mechanism of action |
| Mode of administration |
| Posology (per label) |
| Duration of treatment (per label) |
| Regulatory background | Regulatory designations (orphan, PRIME) | Impact on the perception of innovativeness and level of uncertainty Impact on size of the patient population (current population, extent of future expansion) |
| Type of approval (e.g., conditional, under exceptional circumstances) |
| Current and anticipated additional indications |
| Post-authorisation obligations |
| Study design | Phase of pivotal clinical trial | Impact on willingness to pay (depending on strength of evidence) Impact on the internal and external validity of trial results, and the extent of clinical uncertainties Impact on reimbursement criteria (e.g. restriction to sub-populations) |
| Duration of trial |
| Study design (e.g. single-arm vs. RCT) |
| If RCT, comparator in the control arm (e.g. placebo, SoC) |
| Patient enrolment criteria (inclusion / exclusion criteria) |
| Number of patients enrolled |
| Other design elements of importance (e.g. cross-over, blinding, etc.) |
| Study endpoints | Primary endpoint | For each trial endpoint: specify (objectively) how the endpoint was defined, present its link to patient outcomes (for surrogate endpoints), and describe its relevance to medical practice (e.g. if measured in routine clinical care) |
| Secondary endpoint |
| Exploratory endpoint |
| Impact of treatment on clinical outcomes | Treatment impact on mortality | Specify whether the endpoint was met or not. Where relevant, provide numerical values for key outcomes (e.g., OS, proportion of responders in treatment vs. control group), to help quantify the size of treatment effect |
| Treatment impact on morbidity |
| Treatment impact on quality of life |
| Additional treatment impact not specified above |
| Variability in clinical response across patient subgroups |
| Topline safety results (vs. comparator) |
| Impact of treatment on pharmaceutical costs | Average annual cost per patient | Estimate the direct budget impact of the new treatment and the impact on cost structure over time |
| Comments of annual cost (e.g. one-off cost) |
| Causes of variability of average annual costs (e.g. dosing, induction vs. maintenance phases) |
| Base case cost-effectiveness [COUNTRY-SPECIFIC] |
| Base case budget impact [COUNTRY-SPECIFIC] |
| Impact of treatment on health care resource utilization | Costs offsets (e.g., reduction in healthcare resource consumption) | Identify elements which may heighten or lower the budget impact |
| Auxiliary costs (e.g., hospitalization, ICU use) |
| Impact of treatment on patient and carer experience |  | Adopt a holistic view of treatment benefits; encompass all potential indirect benefits of the treatment, beyond direct health effects |
| Impact of treatment on broader societal costs |  | Adopt a holistic view of treatment benefits |