to-moderate bleeds in a hemophilia patient with inhibitors is very high. When considering the impact of rebleeding over multiple lines of treatment, the rFVIIa-only regimen, versus APCC-containing strategies, may provide cost savings of up to $60,000 annually per patient.

HEMATOLOGICAL DISORDERS—Patient Reported Outcomes

PHM4

COSTS AND CONSEQUENCES OF INADEQUATE COMPLIANCE WITH DEFEROXAMINE THERAPY IN PATIENTS WITH TRANSFUSION-DEPENDENT THALASSEMIA

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OBJECTIVES: Patients with transfusion-dependent thalassemia require chelation to prevent complications from transfusional iron overload. Deferoxamine (DFO) is an effective iron chelator, but must be administered as a subcutaneous or intravenous infusion over 8–12 hours 5–7 times per week leading to poor compliance and/or quality of life in many patients. **METHODS:** We developed a Markov model using data from published studies and other sources to estimate the lifetime incidence and medical care costs of complications of iron overload that are attributable to inadequate compliance with DFO therapy in patients with transfusion-dependent thalassemia. Complications considered included cardiac disease, diabetes, hypogonadism, hypoparathyroidism, hypothyroidism, and death due to cardiac disease. Current compliance with DFO therapy as well as costs of complications were obtained from an analysis of health insurance claims data. Adequate compliance was defined as 260 infusions per year (i.e., five per week). Costs were discounted at 3% annually. **RESULTS:** Current mean DFO use was estimated to be 169 infusions annually. At this level of compliance, 95% of patients are projected to experience cardiac disease during their lifetime, diabetes 46%, hypogonadism 77%, hypoparathyroidism 32%, and hypothyroidism 26%. Cardiac-disease-free life expectancy is projected to be 23 years; overall life expectancy, 28 years. The expected lifetime cost of complications of iron overload is $54,151 per patient. If mean compliance were to increase to 260 infusions per year, the lifetime risk of cardiac disease would decline to 60%, diabetes to 9%, hypogonadism to 47%, and hypothyroidism to 14%. Cardiac-disease-free and overall life expectancy would increase by 22 and 19 years respectively. The discounted expected lifetime costs of complications of iron overload would decline by $30,222. **CONCLUSIONS:** Inadequate compliance with DFO therapy in patients with transfusion-dependent thalassemia results in substantial morbidity and mortality, as well as increased medical care costs associated with complications of iron overload.

PHM5

PILOT STUDY TO ESTABLISH PREFERENCES TOWARDS COAGULATION FACTOR CONCENTRATES USED TO TREAT HAEMOPHILIC PATIENTS WITH INHIBITORS

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OBJECTIVES: Haemophilia is a very expensive disease. This situation becomes extreme when patients develop inhibitors that compromise the effectiveness of treatment, with potential increase of morbidity and mortality. Treatment of haemophilia is the result of interactions between patients, physicians, pharmacists and budget holders, each carrying their own set of preferences. A pilot study was conducted to identify which characteristic of coagulation products are considered more important to treat patients with inhibitors: these characteristics will be included with a price proxy characteristic in a Discrete Choice Experiment, with the objective to elicit preferences and willingness to pay towards treatments of patients with inhibitors. **METHODS:** Eight characteristics were identified during focus groups with patients and clinicians and rated from 0 (not important) to 10 (very important) by 35 people (adult patients, caregivers, physicians, pharmacists). **RESULTS:** the following median (mean) scores were found: "viral safety": 10 (8.9); "time to stop bleeding": 9.5 (9.0); "risk of anamnestic response": 9.0 (8.5); "possibility of undergoing major surgery": 9.0 (8.8); "regular use in prophylaxis": 9.0 (8.4); "time to pain recovery": 9.0 (8.3); "number of injections to stop bleeding": 8.0 (7.9); "time to prepare and give/have the injection": 7.0 (6.6). All groups of respondents considered as more important "viral safety", "possibility of undergoing major surgery", "risk of anamnestic response", "time to stop bleeding", while "time to prepare and give/have the injection" was considered the least important. Different preferences were attributed to "time to pain recovery", considered more important by patients; "regular use in prophylaxis", considered more important by caregivers. **CONCLUSIONS:** Viral safety and effectiveness are considered as the most important characteristics in the treatment of haemophilic patients with inhibitors. Different levels of preferences are present between patients, or their caregivers, and physicians. Understanding these differences is important to guide optimal therapeutic strategies in patients with inhibitors.

HEALTH CARE USE & POLICY

PHP1

A COMPARISON OF CLINICAL TRIAL PARTICIPANTS TO THE GENERAL PATIENT POPULATION

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OBJECTIVE: To determine and quantify the unique characteristics of clinical trial participants in comparison to the general patient population. **METHODS:** Data were obtained from the U.S. National Health and Wellness Survey, an annual, nationally representative, Internet-based study of the health care attitudes and behaviors of non-institutionalized adults age 18+. The sample for analysis included 18,419 respondents who reported a diagnosis of hypertension, high cholesterol, or diabetes. Respondents reported ever participating in a clinical drug trial. They also provided information on demographics, healthcare attitudes, health habits, quality of life measured by the SF-8, and healthcare resource use in the past six months. **RESULTS:** Among respondents diagnosed with hypertension, high cholesterol, or diabetes, 7% (n = 1333) have participated in a clinical drug trial. Clinical trial participants significantly differ from the general patient population in many key characteristics. Clinical trial participants are significantly older (mean age 60.5 versus 55.1, $p < 0.001$) and more educated (college graduates 43% versus 36%, $p < 0.001$). They experience worse physical well-being (sf-8 physical component summary score 43.1 versus 45.3, $p < 0.001$), though are more likely to maintain a healthy diet (50% versus 46%, $p = 0.002$) and less likely to smoke (18% versus 23%, $p < 0.001$). Clinical trial participants are more