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901.HEALTH SERVICES AND QUALITY IMPROVEMENT - NON-MALIGNANT CONDITIONS

Patients' Preferences for Dosing Frequency and out-of-Pocket Cost Associated with Iron Chelators: A Mixed-Methods Pilot Study in the United States

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Patients with sickle cell disease, thalassemia, and other anemias who require regular red blood cell transfusions are at risk of developing iron overload. Excess iron accumulates in tissues and vital organs, such as the liver, heart, and pancreas, causing end organ damage with significant morbidity and higher risk of early mortality. To offset the negative impacts of iron overload, patients are often prescribed iron chelators; however, little is known about what is important to patients when choosing between therapies, including the impact of dosing frequency or cost. The primary objective of our study was to develop and examine the comprehension of a patients' health preference survey regarding the dosing frequency of hypothetical oral iron chelators and the impact of the associated out-of-pocket costs. We hypothesized that patients would prefer iron chelators administered less frequently with some tradeoffs related to cost. The secondary objective was to explore potential reasons for preferring a less frequent dosing schedule to inform a larger-scale online preference study.

A stated-preference survey using a direct elicitation approach was developed and tested in one-on-one, semistructured cognitive interviews with eight adults recruited through L&E Research, a market research firm specializing in healthcare. Patients with sickle cell disease, thalassemia major, or other anemias requiring frequent blood transfusions and who have experience with an iron chelator were invited to participate in an online interview. The survey instrument included a trade-off scenario involving a choice between two hypothetical oral iron chelators. Participants were told to assume that the medicines were just as safe and effective as their current iron chelator and could be taken either twice a day or three times a day. The survey also included questions to help explain preferences and understand the impact of out-of-pocket costs on treatment choice. During the interviews, we followed the "think aloud" approach where participants were asked to read the draft survey aloud, provide answers to the questions, and discuss or explain their interpretations of the information and the motivation behind their answers to selected questions. Open-ended, probing interview questions were used to explore participants' responses further. Qualitative data were generated through interview participants' verbatim accounts of their decision-making. The interviewer and the research team interpreted the qualitative data and made notes and observations to refine the survey instrument between interviews in an iterative process of updating and retesting. Quantitative data were generated through responses to the survey questions and were summarized using descriptive statistics.

The median age of participants was 39 years old (25th percentile: 37 years; 75th percentile: 44 years), and most identified as female (n=7, 87.5%). Five participants were currently taking an oral iron chelator at the time of the interview, but the length of time on treatment varied from less than 6 months (n=2) to 2-5 years (n=3). When asked to choose a hypothetical oral iron chelator, all participants chose oral tablets that would be taken twice daily. The most common reasons for choosing a less frequent dosing schedule included dislike of taking a lot of pills (n=6), not having to remember to take pills (n=5), and not having to carry as many pills with them (n=4). When asked whether they would still choose oral tablets taken twice daily when the monthly out-of-pocket cost was $50, all but one participant switched to oral tablets taken three times a day. Qualitative data suggested participants accepted the hypothetical context of the preference scenarios, and the survey instrument was generally well understood. Participants also reported several other reasons for preferring a less frequent dosing schedule, which will all be included as response options in the final survey.

Although patients indicated a preference for an iron chelator administered less frequently two times a day versus three times a day, choice was affected by the out-of-pocket cost of treatment. The results of our pilot mixed-methods interview study supported patients' comprehension of the health preference survey, which will be administered online to a larger sample to further explore preferences for dosing frequency and out-of-pocket costs of oral iron chelators.

Disclosures Dorling: *Chiesi USA:* Current Employment. **Luzzi:** *Chiesi Farmaceutici S.p.A:* Current Employment. **McMichael:** *RTI Health Solutions:* Current Employment, Other: I am a full time salaried employee of RTI Health Solutions which is a not for profit research institute which conducts research for the pharmaceutical industry. My salary is unconnected to the projects on which I work.. **Kent:** *RTI Health Solutions:* Current Employment, Other: I am a full time salaried employee of RTI Health Solutions which is a not for profit research institute which conducts research for the pharmaceutical industry. My salary is unconnected to the projects on which I work.. **Canty:** *Chiesi:* Current Employment. **Myers:** *RTI Health Solutions:* Current Employment, Other: I am a full time salaried employee of RTI Health Solutions which is a not for profit research institute which conducts research for the pharmaceutical industry. My salary is unconnected to the projects on which I work.. **Badawy:** *Bluebird bio, INC:* Consultancy; *Chiesi, Inc:* Consultancy; *Editas Medicine:* Consultancy; *Bristol-Myers Squibb:* Consultancy; *GBT/Pfizer:* Consultancy; *Forma Therapeutics/Novo Nordisk:* Consultancy; *Vertex Pharmaceuticals Inc:* Consultancy.

Figure 1 **Tradeoff Scenario Comparing Two Hypothetical Oral Iron Chelators**

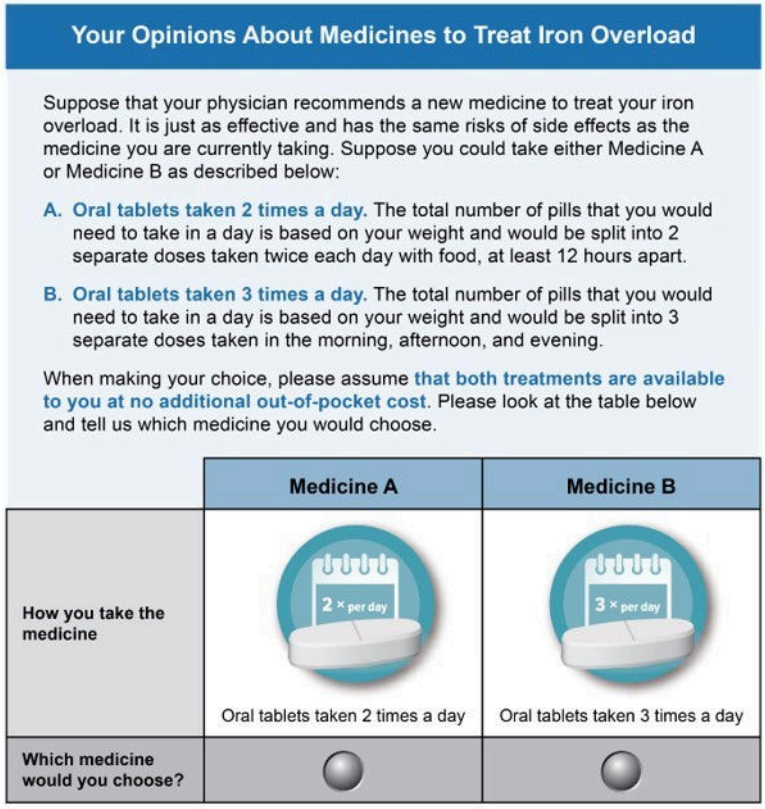

Figure 1

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