haematologica
Journal of the Ferrata Storti Foundation

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Received: March 7, 2025.
Accepted: July 3, 2025.

Citation: Fabian Lang, Zack Pemberton-Whiteley, Joannie Clements, Cristina Ruiz, Delphine Rea, Lisa Machado, Naoto Takahashi, Sung-Ho Moon, Andrew Grigg, Cornelia Borowczak, Peter Schuld, Pauline Frank,Cristina Constantinescu, Carla Boquimpani and Jorge E. Cortes. Improving chronic myeloid leukemia management and quality of life: patient and physician survey on unmet needs from the CML SUN survey.
Haematologica. 2025 July 10. doi: 10.3324/haematol.2025.287772 [Epub ahead of print]

TITLE

Improving chronic myeloid leukemia management and quality of life: patient and physician survey on unmet needs from the CML SUN survey

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RUNNING TITLE: CML SUN: Survey on unmet needs in CML

Disclosures: All authors received nonfinancial support (assistance with manuscript preparation) from Nucleus Global, which received funding from Novartis Pharmaceuticals Corporation. F.L. received honoraria and consulting fees from Bristol Myers Squibb, Incyte, Celgene, and Novartis and research funding from Novartis. Z.P.-W. is a consultant and member of the Board of Directors or an advisory committee for Acute Leukemia Advocates Network and Workgroup of European Cancer Patient Advocacy Networks, is a member of the Board of Directors or an advisory committee for CML Advocates Network, is a consultant, received organizational grant funding, and is a member of the speakers bureau for AbbVie, Agios, Amgen, Astellas, Autolus, Bristol Myers Squibb, Celgene, Daiichi Sankyo, Gilead, Incyte, Jazz, Janssen, Kite, Kyowa Kirin, Mallinckrodt, Novartis, Pfizer, Roche, Servier, and Takeda, and is currently employed by Novartis. J.C. received honoraria and consulting fees from Novartis, Pfizer, and Takeda, grant funding for the CML Buster Foundation from Pfizer, scholarships to attend meetings from CML Advocates Network NPO and the Max Foundation, participated in advisory board meetings with Novartis, has volunteer leadership roles with the CML Buster Foundation and the Leukemia and Lymphoma Society, and has received medication assistance through Patient Services, Inc. C.R. received honoraria from Novartis and Pfizer, travel costs from Novartis, and was a volunteer patient advocate on a panel and subsequent manuscript with the US Food and Drug Administration. D.R. received honoraria from and is a member of the Board of Directors or an advisory committee for Novartis, Incyte, and Pfizer and provided consultancy for Novartis. L.M. received honoraria from Novartis. N.T. received research funding and honoraria from and is a member of the speakers bureau and Board of Directors or advisory committee for Novartis, Pfizer, and Otsuka. S.-H.M. reports no conflicts. A.G. received consulting fees from Novartis. C. Borowczak received honoraria from and is a member of the Board of Directors or an advisory committee for Novartis. P.S. and P.F. are employed by Novartis. C.C. is employed by IPSOS, a consulting company paid by Novartis. C. Boquimpani is a member of the speakers bureau for Novartis, Janssen, and Pint Pharma, and is a member of the Board of Directors or advisory committee for Novartis and Janssen. J.E.C. received research support and consulting fees from Novartis, Pfizer, Sun Pharma, and Takeda, honoraria from Novartis, Pfizer, Terns Pharmaceuticals, Incyte, and Takeda, and has stock options in Bio-Path Holdings.

Acknowledgments

We thank all patient and physician participants for their involvement in this study. Medical writing support was provided by Michelle Chadwick, PhD, and Kenneth Maus, PhD, of Nucleus Global, Inc, and was funded by Novartis Pharma AG. This manuscript was developed in accordance with Good Publication Practice (GPP3) guidelines. Authors had full control of the content and made the final decision on all aspects of this publication.

Statement of prior presentation:

Some of these data were presented in poster form at the EHA2023 Hybrid Congress, Frankfurt, Germany, 8-11 June 2023 and at the SOHO2023 Hybrid Congress, Houston, TX, 6-9 September 2023.

Data sharing statement:

Novartis is committed to sharing with qualified external researchers, access to patient-level data and supporting clinical documents from eligible studies. These requests are reviewed and approved by an independent review panel on the basis of scientific merit. All data provided is anonymized to respect the privacy of patients who have participated in the study in line with applicable laws and regulations. This study data availability is according to the criteria and process described on www.clinicalstudydatarequest.com.

Abstract

For patients with chronic myeloid leukemia in chronic phase (CML-CP), disease management, treatment experiences, and decisions around switching therapies due to resistance or intolerance can have significant impacts on their lives. Experiences and perspectives regarding the roles of patients and treating physicians in shared decision-making are poorly understood. The CML Survey on Unmet Needs (CML SUN), the largest CML survey to date, was initiated to gather insights from patients with CML-CP and physicians on disease management, including treatment goals, decision-making, satisfaction, tolerability and the impact of CML on daily life. The survey was deployed in 11 countries with 361 patient and 198 physician participants and comprised separate questionnaires for each group. Results indicated that nearly three-quarters of physicians saw themselves as the ultimate initial treatment decision-makers; only a quarter of patients reported that these decisions were discussed and decided together with their physician. Nearly half of physicians reported making treatment decisions across all lines of therapy with little to no input from the patient. Disparities between patient and physician opinions were observed regarding treatment goals, especially the balance between efficacy and tolerability. The CML SUN highlights the need for improvements in communication about treatment options and the importance of shared treatment decision-making to unify treatment goals.

Introduction

Survival among patients diagnosed with chronic myeloid leukemia in chronic phase (CML-CP) has markedly improved with the use of tyrosine kinase inhibitors (TKIs) such that most patients now have a life expectancy close to that of the general population.[1-3] Approximately a quarter of patients with CML-CP with prolonged molecular remission are able to successfully discontinue TKI treatment.[4] However, the majority of patients will require lifelong treatment, which may have a substantial impact on their quality of life (QOL).[1-3] Patients report an impact on sleep, memory, mental state, mood, work, general activities, and sexual relations.[5] When choosing a new therapy, overall patient health, presence of specific comorbidities, treatment goals, and prior therapies (for patients receiving their second or later TKI) are important considerations both for disease control and QOL. Therefore, an optimized patient-physician relationship built on good communication and shared decision-making is an essential part of every patient's journey.[5]

Despite the fact that TKIs have been the cornerstone of CML treatment for >20 years, starting with the approval of imatinib in 2001 in the United States, there remains a critical gap in understanding the real-world experiences of patients and physicians.[6] While clinical milestones and molecular response rates are widely studied, the nuances of patient-physician communication, treatment satisfaction, and shared decision-making are often overlooked. These aspects, however, play a fundamental role in adherence, QOL, and long-term treatment success. In recent years, some studies have sought to gather insights on patient experience and treatment goals.[5,7] A 2020 synthesis of qualitative studies reported that CML patients receiving a TKI experience significant treatment-related side effects and alterations to their mental and physical wellness.[7] In addition, this study noted that patients may not have received sufficient information about their disease and may feel unprepared to participate in treatment decisions.[7] Another study reported low levels of treatment satisfaction, specifically around eliminating CML, preventing its recurrence, and returning to a normal life, which is particularly important given the relationship between satisfaction, QOL, and treatment adherence.[5] These earlier studies, although valuable, are limited by their inclusion of only a few countries and their focus primarily on the patient's perspective without a comparison with that of the treating physicians.[5,7] With the increasing prevalence of CML, there is a need for more data on both patient and physician perspectives of the respective priorities in CML management to enable a unified understanding of factors impacting adherence, QOL and optimal disease outcomes. To address these knowledge gaps, the Chronic Myeloid Leukemia Survey on Unmet Needs (CML SUN) was conducted.

Methods

The CML SUN used a mixed-methods research approach with exploratory sequential design in which a qualitative phase was followed by a quantitative phase. Data analysis and integration of patient and physician responses were conducted after each phase (**Supplementary data Figure S1**). An external steering committee composed of physicians and advocates of patients with CML provided guidance throughout the research and input into the fieldwork material. Findings from the qualitative phase have been previously reported in abstract form[8] and the results from the quantitative phase are reported here.

Qualitative interviews and quantitative surveys

Qualitative interviews were conducted using semi-structured discussion guides developed from an assessment of patient-focused literature in CML as well as the collective insights of patients with CML, patient advocates and treating physicians involved in the research.[8]

In the quantitative phase, the results of the qualitative interviews were used to inform topics and questions for the quantitative online surveys. Further details on qualitative and quantitative assessments are described in the **Supplementary Methods.**

Cognitive testing

Cognitive testing was performed to minimize ambiguity and ensure that respondents understood the survey questions and response options, recalled the information being asked, did not miss any important questions or response options, and did not become overwhelmed. A sample of target respondents completed cognitive testing via video conferencing with screen sharing prior to deployment of the online surveys. Target respondents were 6 patients and 3 physicians in the US, Canada, and the UK who met the inclusion criteria for survey participation (**Supplementary data Table S1**). Results of cognitive interviews were analyzed by the research team, and the feedback was used to adapt the survey content. The revised surveys were shared with the steering committee before survey implementation.

Recruitment

The target population for CML SUN were patients with CML-CP and physicians who treated patients with CML. Eligibility criteria are described in **Supplementary data Table S1**. Patients were recruited through patient databases, physician and nurse referrals, and patient advocacy groups. Physicians were recruited via online physician panels. In appreciation for their time, patients and physicians were offered compensation in line with fair market value, including options to donate to an international patient organization or decline compensation.

Analysis

For analysis of the qualitative and quantitative data, transcripts and descriptive statistics were used, respectively. Continuous variables were summarized using means and medians. Reported results were derived from survey questions described in **Supplementary Methods.**

Ethics

Answers from both patients and physicians were anonymized to ensure confidentiality and avoid bias during data collection and analysis. The surveys conducted were reviewed by a central Institutional Review Board (IRB). Two exemptions (one for qualitative phase and one for quantitative phase) were granted by Pearl IRB. Eligible respondents gave electronic informed consent prior to participation. CML SUN was conducted in accordance with the Declaration of Helsinki and all standards of Good Clinical Practice (GCP), as well as US-healthcare personal data protection law (HIPAA, Health Insurance Portability and Accountability Act), as well as European laws and regulations (GDPR, General Data Protection Regulation).

Results

A total of 361 patients with CML-CP and 198 physicians in 11 countries participated in the online survey from November 2022 to March 2023 (**Supplementary data Table S2**). Patients had a median age of 45 years (range, 19-82 years), most (66%) were on their second TKI, and 56% were female (**Supplementary data Table S3**). Patients reported public insurance only (33%) or public insurance plus private supplementary insurance (27%), with 17% of patients reporting private insurance coverage only (**Supplementary data Table S3**). Physicians had been in practice a median of 18 years (range, 3-31 years) and spent a median of 90% of their time caring for patients. Most physician respondents (59%) practiced at university teaching hospitals, 17% practiced at community or general hospitals, 15% practiced at dedicated cancer centers, 8% were in solo or group practice, and 1% practiced in a general hospital. Half of their patients were currently receiving their first TKI, while the other half were receiving their second or later TKI (**Supplementary data Table S3**).

Information sharing at diagnosis

The top 3 areas of information patients wanted to receive at diagnosis were (a) potential side effects of treatment, (b) how the disease will progress and impact their life, and (c) the CML treatment that will be given to them, what it is, how to take it, and what to expect from it (**Supplementary data Figure S2**). Approximately 70% of physicians reported providing information on these topics but only about half of patients reported receiving all the information they needed, and a third wished they had received more information at diagnosis.

Information sharing at switching treatment

Patients switching treatments similarly wanted information about potential side effects and how to manage them, as well as how to understand test results, available treatment options, and how to contact their care team. However, only about 60% of physicians reported offering information on management of potential side effects and reasons for treatment switching. Only half of physicians reported providing information about specifically what to do if patients experienced intolerable side effects and only 36% provided a way to contact the care team if the patient has any questions. Approximately a third reported providing information on other treatment options in case the second treatment was not successful and only 29% reported providing patients with information on how to understand their test results.

Treatment goals

Although patients and physicians generally align on the importance of having manageable side effects and maintaining or improving QOL, physicians ranked achievement of major molecular response and deep molecular response higher than patients, regardless of the number of prior TKIs patients received (**Figure 1**).

Shared treatment decision-making

Patients reported that they want to receive information about treatment options at diagnosis and if a treatment switch is needed (**Supplementary data Figure S2**), but approximately 40% reported receiving information about only one TKI from their physician regardless of number of prior TKIs. Furthermore, only about 25% of patients felt that treatment decisions were discussed and decided on together with their physicians (**Figure 2**).

Most physicians thought that patients should be more involved in treatment decisions and reported that their patients have an active role in determining their treatment; most (74%) saw themselves as the ultimate decision-maker (**Figure 3**). Approximately half reported making treatment decisions with little to no input from the patient (**Figure 2**). Regardless of number of prior TKIs, more than half of physicians reported presenting only one treatment option, mostly to avoid overwhelming patients and/or because they feel that the patients may not understand all the details and thus cannot make an informed decision (**Figure 4**). Physicians perceived that less than a quarter of patients understand information they were given; in contrast, most patients report understanding all the information they received (**Supplementary data Figure S3**).

Burden of disease: patient perspective

While most patients were satisfied with current treatments (**Figure 3**), many reported that the diagnosis and treatment caused worry and stress, and made them physically and emotionally fatigued, thus limiting their personal and social lives and adversely affecting QOL (**Figure 5**). Some patients expressed dissatisfaction with the impact of side effects on their finances and occupational, social, and educational lives (**Figure 6**). The top patient fears were that their treatment would stop working, have a long-term impact on their body, or produce side effects (**Supplementary data Figure S4**).

Treatment switching

When asked who initiated discussions of treatment change, patients and physicians agreed that physicians usually initiate the discussion (**Supplementary data Figure S5**). Lack of efficacy and intolerance were reported by both groups as the most common reasons for treatment switching (**Supplementary data Figure S6**). The most important factors for patients when considering a treatment change were to have a normal life expectancy and maintain or improve QOL, while achieving molecular response, helping long-term survival, and having manageable side effects were the most important factors for physicians. Treatment logistics were among the least important factors for both patients and physicians (**Supplementary data Figures S7 and S8**).

Side effects

When asked who initiated the discussions around side effects, up to 1 in 5 patients reported that they noticed side effects but did not tell their doctor unless specifically asked (**Supplementary data Figure S9**). Patients were also asked what their doctor did when they informed them of side effects (**Figure 7**). Notably, less than half reported feeling empathy from their physician and only a third of patients reported being given medication to manage side effects. About 20% of patients reported that their physician did not think that their side effects were serious and expected them to continue their current therapy.

Adherence

Both patients and physicians reported forgetfulness and side effects as the primary reasons for missed doses (**Supplementary data Figure S10**).

Digital healthcare solutions for managing CML

Patients ranked the most helpful digital tools to assist with managing CML as digital solutions to aid their diet, exercise, and well-being (73%), save important information between doctor visits (73%), get in touch with other patients with CML (72%), track and help manage symptoms and side effects (72%), and remind them of medical appointments/tests (71%) (**Supplementary data Figure S11**). Patients ranked the least helpful digital tools as those which remind them when to take medication (19%), look up the drugs that are used to treat CML (15%), look up information about clinical trials (13%), remind them of medical appointments/tests (13%), and remote consultation with healthcare professionals, supporting communication and information sharing (12%) (**Supplementary data Figure S11**).

Discussion

This study underscores the need to re-evaluate how treatment success is defined—not solely through molecular response, but also through patient-centered measures such as QOL, treatment burden, and informed decision-making. The disconnect between physician and patient priorities has profound implications for treatment adherence and long-term outcomes, highlighting areas that could inform future improvements in clinical practice and patient care. While the results underscore some disparities in prioritization and communication, they also reveal opportunities to enhance shared decision-making and align treatment goals more effectively. As the medical community continues to advance CML treatment options, optimizing patient-physician communication and ensuring alignment in treatment expectations are equally critical.

One key finding was the perception by patients of their physician's recognition of side effects and their impact on QOL. Approximately 1 in 5 patients did not tell their doctor about side effects unless specifically asked. Less than half felt their doctor showed empathy and asked sufficient questions about side effects. This is consistent with other studies indicating that physicians tend to underestimate the symptoms experienced by patients.[9] Unlike safety, which is based on the physician's judgment,[10] tolerability reflects the extent to which adverse events affect the ability or desire of patients to adhere to therapy.[11] Noting that side effects were a main factor impacting adherence, a failure of communication may lead to suboptimal treatment outcomes. Hence, this study suggests that an environment in which the patient feels comfortable in discussing treatment and their doctor routinely initiates such discussion and takes their concerns seriously is not always the case but should be the aim in all such interactions. This communication deficiency could be addressed by pharmacy teams, teaching and involvement of advanced practitioners, and defined roles for nursing in the follow-up process, which have been shown to foster greater identification of QOL changes and low-grade side effects among varied patient populations.[12-14]

Patients placed more emphasis on stopping/slowing disease progression and physicians placed higher emphasis on achievement of molecular responses. Similarly, achieving normal life expectancy was the predominant factor for patients when considering a treatment switch, whereas for physicians it was molecular response. However, this may not represent misalignment of treatment goals in all instances, as physicians may view molecular response as a surrogate marker for reduced risk of disease progression. While these perspectives suggest a shared overarching goal, patient education around the importance of molecular response milestones[15] can be improved and may lead to better adherence and foster a stronger alignment between clinical objectives and patient understanding.

The findings also highlight gaps in the information exchange at critical points in the treatment journey. About 70% of physicians reported providing information on topics ranked by patients as most important to receive at diagnosis. However, despite physicians’ reported efforts to provide key information, many patients indicated unmet informational needs, particularly regarding treatment options, side effect management, and strategies for handling treatment transitions. Addressing these gaps could improve patient empowerment and satisfaction. Tailoring informational resources to individual preferences and different literacy levels and integrating tools like visual aids or decision support systems may help bridge this gap and improve patient engagement. Indeed, the CML SUN found that a digital tool to track medical appointments and tests was ranked as helpful or very helpful by 71% of patients—placing it in the top 5 tools—yet 13% rated it not very helpful or not at all helpful, placing it simultaneously among the bottom 5. This variability highlights that a one-size-fits-all approach may fall short in meeting patients' diverse informational needs.

Another key insight is the disparity in perceptions of treatment decision-making. Most physicians agreed with the statement that while patient input is valued, the responsibility of the final decision ultimately lies with physicians. However, patients expressed a desire for more involvement in these decisions. This disconnect may stem from physicians underestimating patients' capacity to understand treatment details even though more than half of patients surveyed reported understanding all the information they received. While the level of understanding may depend on both the complexity of the information provided and the way it is presented, physician perception of a lack of patient understanding might be a limiting factor in involving patients in decision-making and may create a new communication gap or widen an existing one. Each patient has a different level of motivation and comprehension when it comes to disease awareness and treatment decisions, and physicians must take this into account during patient interactions as well. For example, some patients come to their physician with preconceived notions based on their own research, which may influence the treatment decision. Thus, there is a need for balanced and inclusive discussions to address the gap between patient expectations and professional medical advice, and for tailored patient education and support to ensure that patients feel heard and involved in informed decision-making.

Results from this study support the findings of prior patient-focused research, including the negative impact of treatment on QOL and adherence, and a need for better patient-physician communication and understanding of patient-specific goals.[5,7] Previous surveys generally included smaller samples, primarily captured patients' views, and were focused on individual countries or a small number of countries. The CML SUN study expands on these findings, including not only more patients from more countries but also bringing the physicians' perspectives to provide a more holistic view.[5,7]

Limitations of this study include the potential for recall and selection bias. Some responses may have been impacted by limited recall and/or influenced by more recent experiences, such as first-line insights and motivations for treatment selection at diagnosis. Although we aimed for as diverse patient recruitment channels as possible via physicians/nurses, patient organizations, and online patient panels, the voluntary nature of the surveys may have resulted in self-selection bias, possibly leading to a higher level of both patient involvement and motivation in the study population. Patients who volunteered to participate were likely to have greater health literacy and be more active in the management of their CML than the general population of patients with CML. Physicians, in contrast, may have been alluding to a more diverse patient population when reflecting about their experience and thoughts. Indeed, practice setting (e.g., university teaching hospital vs community cancer center) and volume of patients with CML treated may also influence physician perspectives and impact findings. Additionally, the surveys were conducted independently among two distinct groups—patients and physicians—rather than pairing the responses of individual patients with their treating physicians, which may limit the depth of potential conclusions. Furthermore, stratification by geographic area (including regional restrictions and regulatory differences), communication culture and expectations, and healthcare systems may reveal region-specific trends and shed light on how such factors influence patient-physician dynamics. This is particularly relevant when considering disparities in treatment access and physician expertise driven by the factors outlined above.

In conclusion, this study underscores the critical role of effective communication and shared decision-making in CML management. Specifically, there is a perceived need by patients for greater involvement in treatment decisions and more empathy in dealing with both specific side effects of treatment and other impacts of the CML diagnosis on their QOL and financial security. The likely outcome is that this will enhance decision-making about the most appropriate and optimal TKI at the start of therapy and at the time of treatment switch due to resistance or intolerance, while also enhancing adherence and ultimately improving outcomes. By addressing the informational and relational gaps identified, patients and physicians can collaborate more effectively to optimize treatment success. These findings emphasize the need for a patient-centered approach that balances safety and efficacy goals while fostering mutual understanding, such as a care team or specialist nurses for more thorough follow-up and patient-friendly educational material. For instance, specialist nurse programs have been shown to significantly reduce emergency admissions and improve outcomes in diverse clinical settings.[12-13] Similarly, the use of patient-friendly educational materials, including digital tools, has been associated with improved treatment adherence, satisfaction, and clinical outcomes across multiple disease areas.[16-17] The results of the CML SUN can also be used by healthcare institutions, patient advocacy groups, industry leaders, and other key stakeholders in CML-CP management to achieve better outcomes through shared decision-making.

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Figure Legends

Figure 1. Top 5 treatment goals by number of TKIs[a]. Top treatment goals of patients and physicians by number of TKIs.

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DMR, deep molecular response; HR, hematologic response; MMR, major molecular response; QOL, quality of life; TKI, tyrosine kinase inhibitor; WBC, white blood cell.

[a] Patients ranked their 3 most important treatment goals by number of prior TKIs; physicians selected any goals that they have by number of prior TKIs.

Figure 2. Patient and physician input on treatment selection. Roles in treatment decision-making as assessed by proportion of patients and physicians by number of TKIs.

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TKI, tyrosine kinase inhibitor.

Figure 3. Physicians' perspectives regarding different management of CML-CP. Physicians ranked items on a scale of 1 to 7, where 1 means strongly disagree and 7 means strongly agree (disagree, 1-3; neither agree nor disagree, 4; agree, 5-7).

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CML, chronic myeloid leukemia; CP, chronic phase.

Figure 4. Number of treatment options presented to patients by physicians. The number of treatment options for which physicians give details and the reasons for giving details about just 1 treatment, as assessed by the proportion of physicians by number of TKIs.

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NA, no answer; TKI, tyrosine kinase inhibitor.

[a] <5% of respondents chose the responses regardless of number of TKIs: other (reason not listed as an option) provided just the details of the treatments available for a given number of TKIs, and provided just the details of the treatments available as per the guidelines/protocols.

[b] Base: respondents who mentioned giving 1 treatment option per number of TKIs.

Figure 5. Patients' perceptions of how CML-CP treatment affects their lives. Patients were asked how much they agreed with statements around how their current TKI treatment has impacted their lives and answered from the following choices: strongly or somewhat agree, strongly or somewhat disagree, uncertain, or not applicable.

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CML, chronic myeloid leukemia; CP, chronic phase.

Figure 6. Patients' satisfaction with their current treatment. Patients ranked items on a scale of 1 to 10, where 0 to 3 is dissatisfied, 4 to 6 is neither dissatisfied nor satisfied, and 7 to 10 is satisfied.

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QOL, quality of life.

Figure 7. Response of physicians to side effects from the patient perspective. Proportion of patients with different responses from their physicians about side effects when recalling side effects on their first and second TKI.

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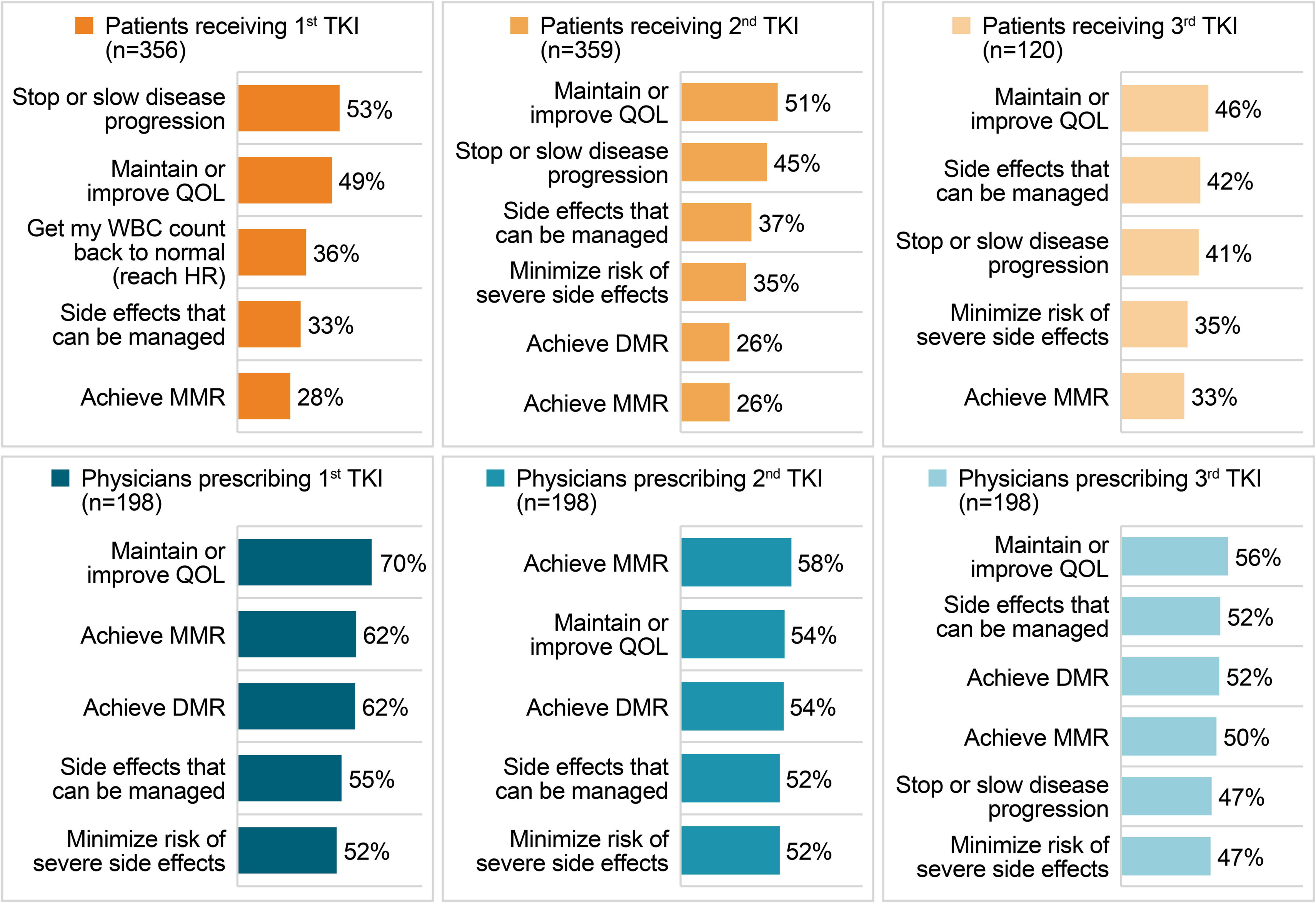
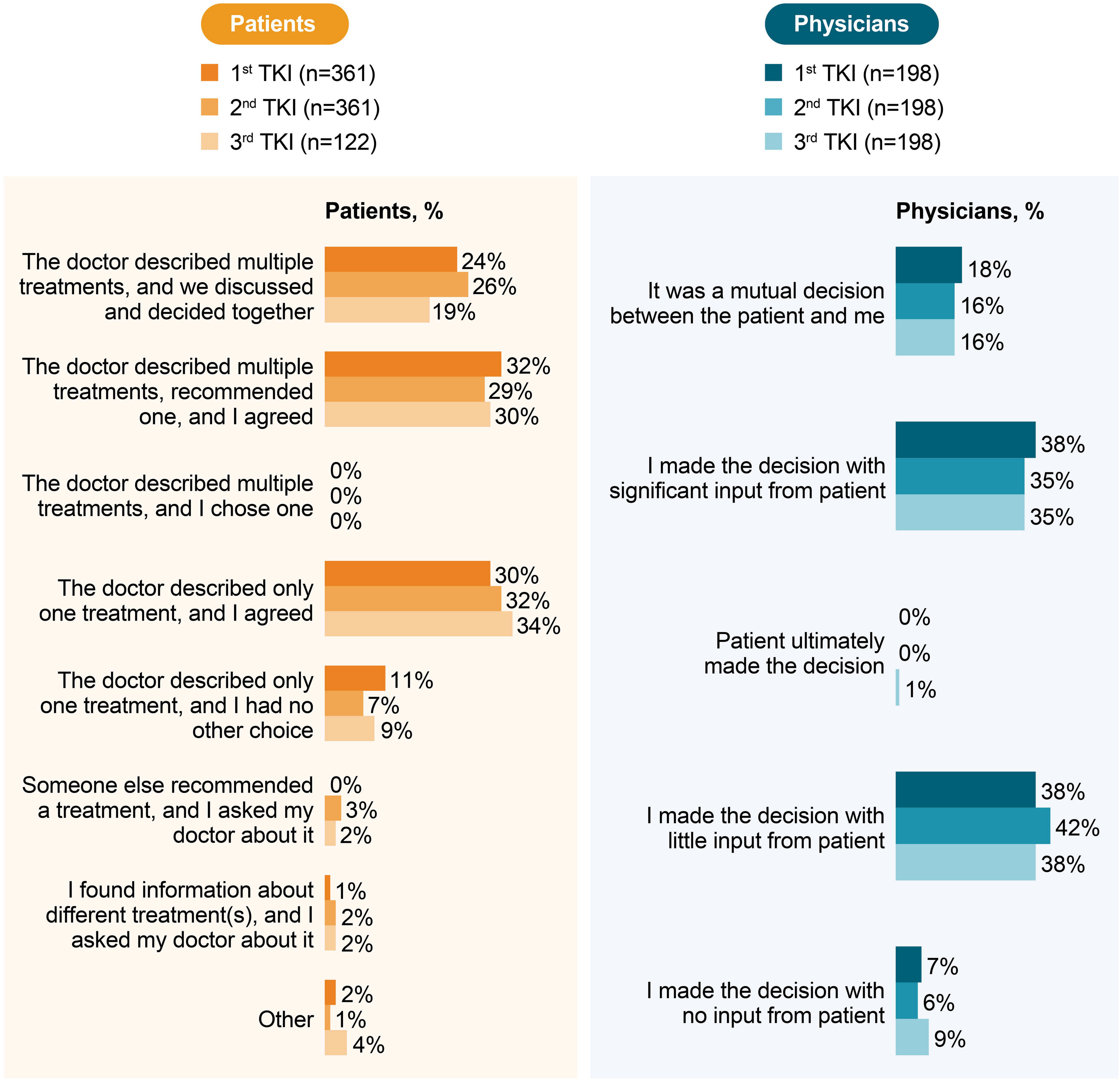
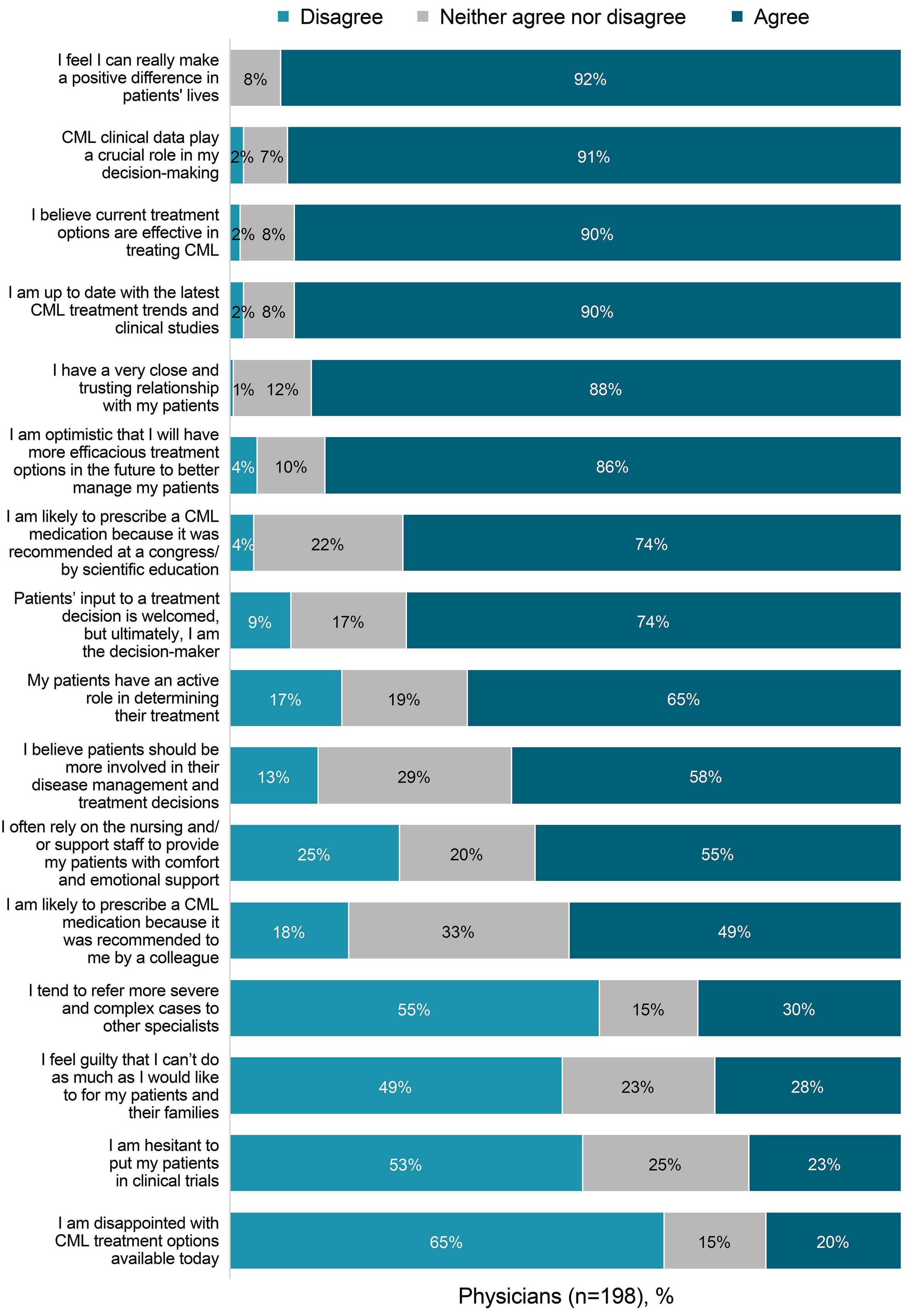
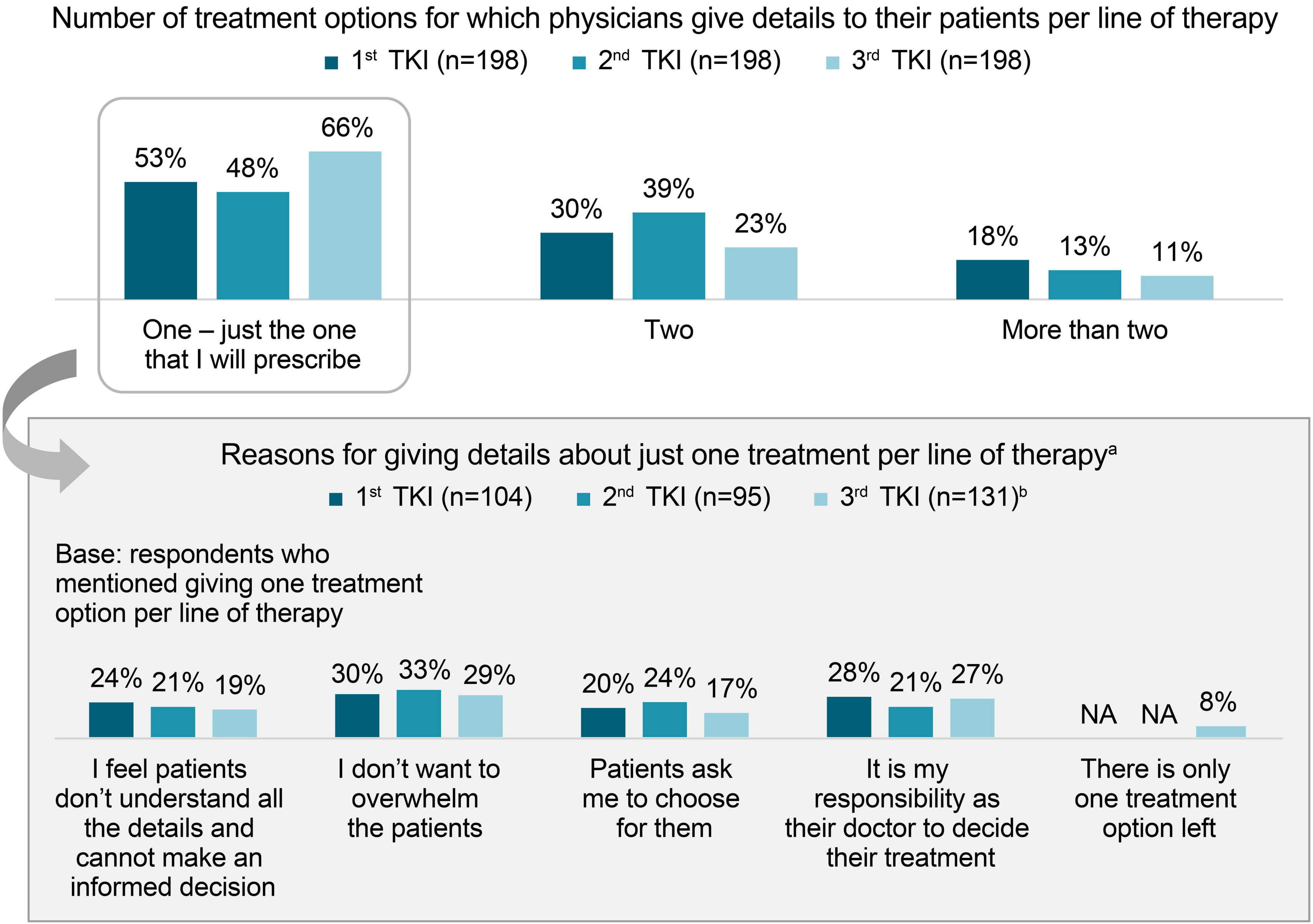
[a] Includes patients who switched treatment because of side effects and reported informing their physician about their side effects.

TKI, tyrosine kinase inhibitor.


Patients receiving 1st TKI (n=356)
Stop or slow disease progression 53%
Maintain or improve QOL 49%
Get my WBC count back to normal (reach HR) 36%
Side effects that can be managed 33%
Achieve MMR 28%
Patients receiving 2nd TKI (n=359)
Maintain or improve QOL 51%
Stop or slow disease progression 45%
Side effects that can be managed 37%
Minimize risk of severe side effects 35%
Achieve DMR 26%
Achieve MMR 26%
Patients receiving 3rd TKI (n=120)
Maintain or improve QOL 46%
Side effects that can be managed 42%
Stop or slow disease progression 41%
Minimize risk of severe side effects 35%
Achieve MMR 33%
Physicians prescribing 1st TKI (n=198)
Maintain or improve QOL 70%
Achieve MMR 62%
Achieve DMR 62%
Side effects that can be managed 55%
Minimize risk of severe side effects 52%
Physicians prescribing 2nd TKI (n=198)
Achieve MMR 58%
Maintain or improve QOL 54%
Achieve DMR 54%
Side effects that can be managed 52%
Minimize risk of severe side effects 52%
Physicians prescribing 3rd TKI (n=198)
Maintain or improve QOL 56%
Side effects that can be managed 52%
Achieve DMR 52%
Achieve MMR 50%
Stop or slow disease progression 47%
Minimize risk of severe side effects 47%

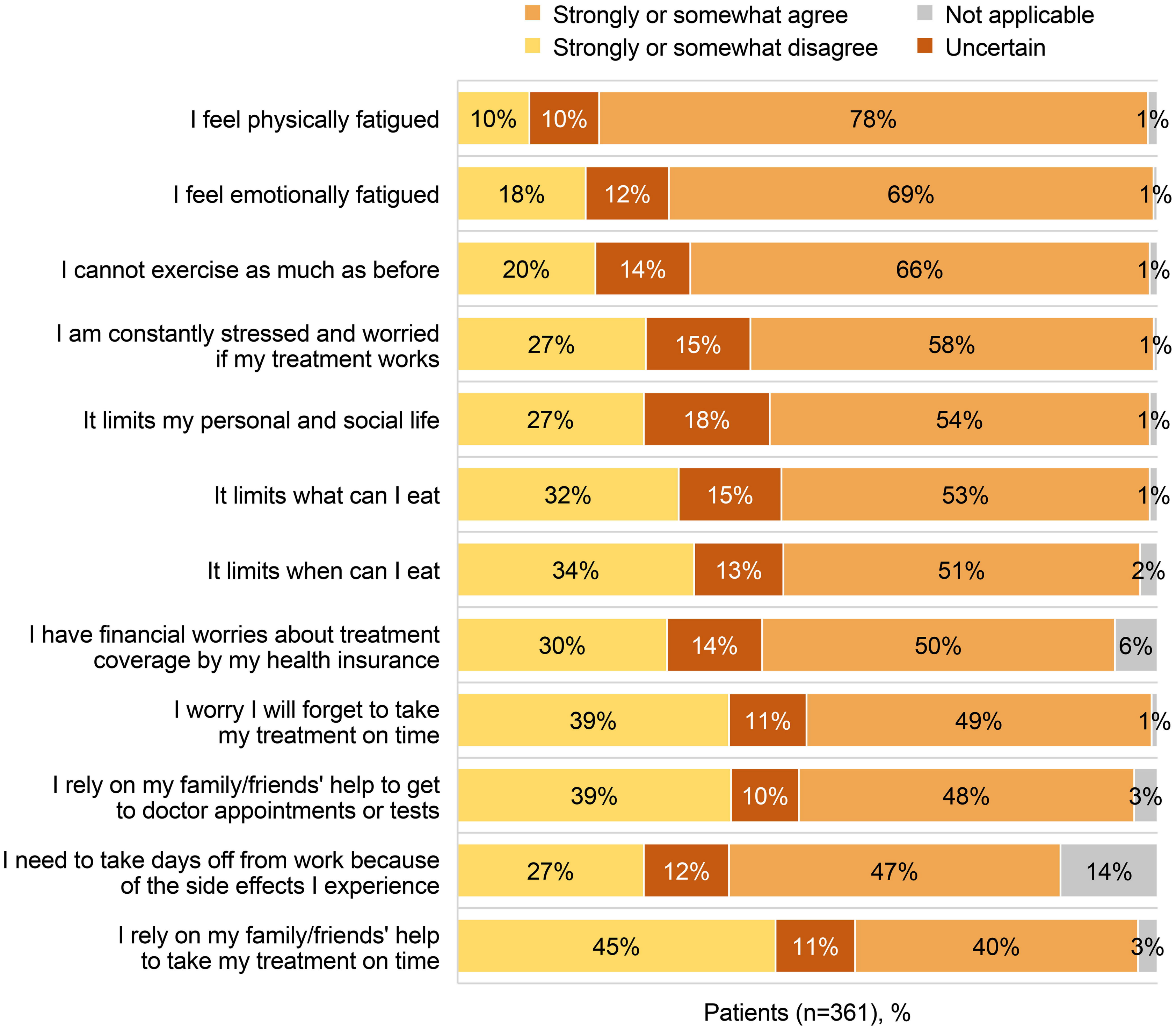

Patients
1st TKI (n=361)
2nd TKI (n=361)
3rd TKI (n=122)
Physicians
1st TKI (n=198)
2nd TKI (n=198)
3rd TKI (n=198)
Patients, %
The doctor described multiple treatments, and we discussed and decided together
24%
26%
19%
The doctor described multiple treatments, recommended one, and I agreed
32%
29%
30%
The doctor described multiple treatments, and I chose one
0%
0%
0%
The doctor described only one treatment, and I agreed
30%
32%
34%
The doctor described only one treatment, and I had no other choice
11%
7%
9%
Someone else recommended a treatment, and I asked my doctor about it
0%
3%
2%
I found information about different treatment(s), and I asked my doctor about it
1%
2%
2%
Other
2%
1%
4%
Physicians, %
It was a mutual decision between the patient and me
18%
16%
16%
I made the decision with significant input from patient
38%
35%
35%
Patient ultimately made the decision
0%
0%
1%
I made the decision with little input from patient
38%
42%
38%
I made the decision with no input from patient
7%
6%
9%

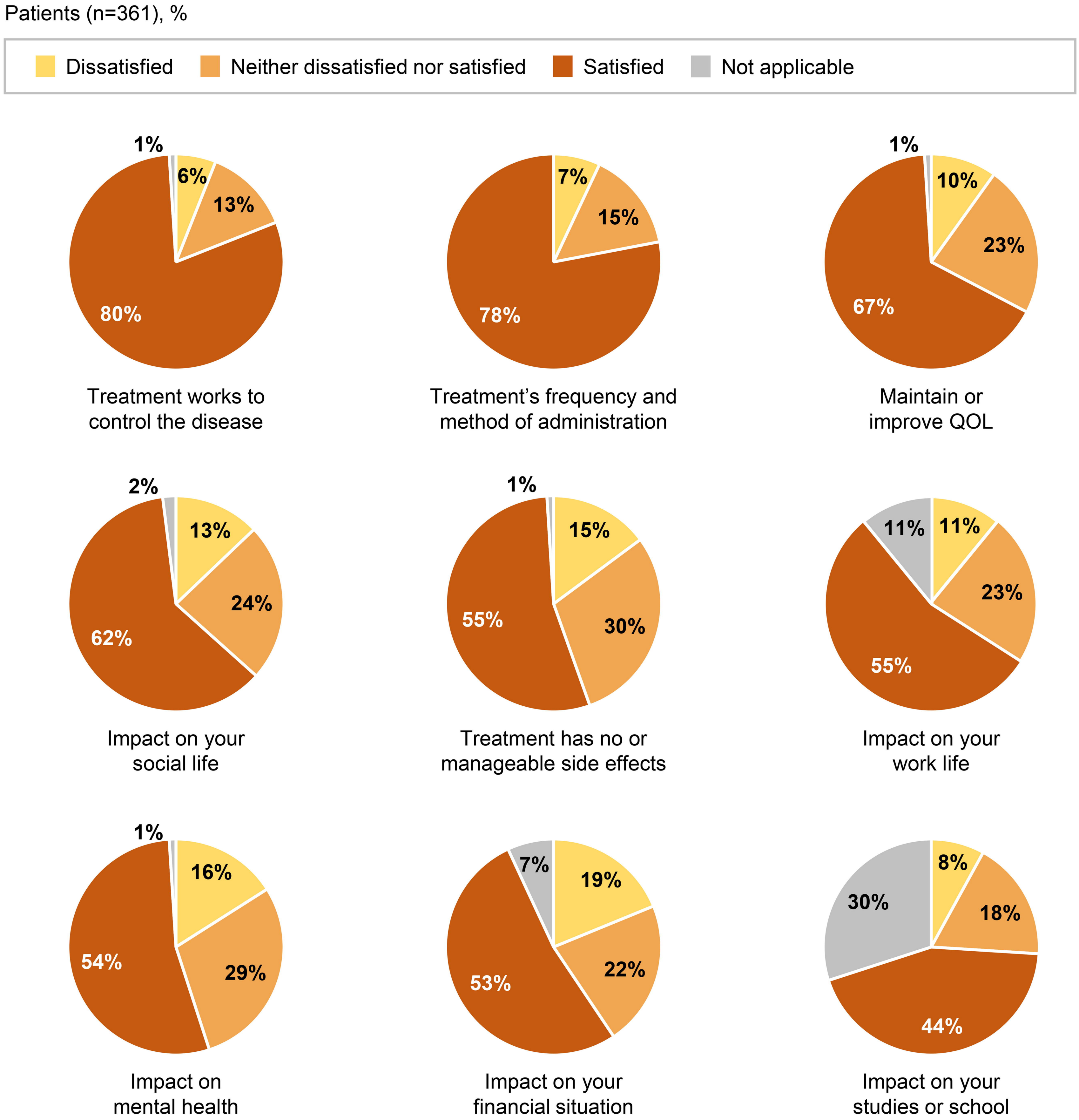

Disagree
Neither agree nor disagree
Agree
I feel I can really make a positive difference in patients' lives
8%
92%
CML clinical data play a crucial role in my decision-making
2%
7%
91%
I believe current treatment options are effective in treating CML
2%
8%
90%
I am up to date with the latest CML treatment trends and clinical studies
2%
8%
90%
I have a very close and trusting relationship with my patients
1%
12%
88%
I am optimistic that I will have more efficacious treatment options in the future to better manage my patients
4%
10%
86%
I am likely to prescribe a CML medication because it was recommended at a congress/ by scientific education
4%
22%
74%
Patients' input to a treatment decision is welcomed, but ultimately, I am the decision-maker
9%
17%
74%
My patients have an active role in determining their treatment
17%
19%
65%
I believe patients should be more involved in their disease management and treatment decisions
13%
29%
58%
I often rely on the nursing and/ or support staff to provide my patients with comfort and emotional support
25%
20%
55%
I am likely to prescribe a CML medication because it was recommended to me by a colleague
18%
33%
49%
I tend to refer more severe and complex cases to other specialists
55%
15%
30%
I feel guilty that I can't do as much as I would like to for my patients and their families
49%
23%
28%
I am hesitant to put my patients in clinical trials
53%
25%
23%
I am disappointed with CML treatment options available today
65%
15%
20%
Physicians (n=198), %



Number of treatment options for which physicians give details to their patients per line of therapy

Reasons for giving details about just one treatment per line of therapy[a]


Strongly or somewhat agree
Not applicable
Strongly or somewhat disagree
Uncertain
I feel physically fatigued
10%
10%
78%
1%
I feel emotionally fatigued
18%
12%
69%
1%
I cannot exercise as much as before
20%
14%
66%
1%
I am constantly stressed and worried if my treatment works
27%
15%
58%
1%
It limits my personal and social life
27%
18%
54%
1%
It limits what can I eat
32%
15%
53%
1%
It limits when can I eat
34%
13%
51%
2%
I have financial worries about treatment coverage by my health insurance
30%
14%
50%
6%
I worry I will forget to take my treatment on time
39%
11%
49%
1%
I rely on my family/friends' help to get to doctor appointments or tests
39%
10%
48%
3%
I need to take days off from work because of the side effects I experience
27%
12%
47%
14%
I rely on my family/friends' help to take my treatment on time
45%
11%
40%
3%
Patients (n=361), %



Treatment works to control the disease

Treatment's frequency and method of administration

Maintain or improve QOL

Impact on your social life

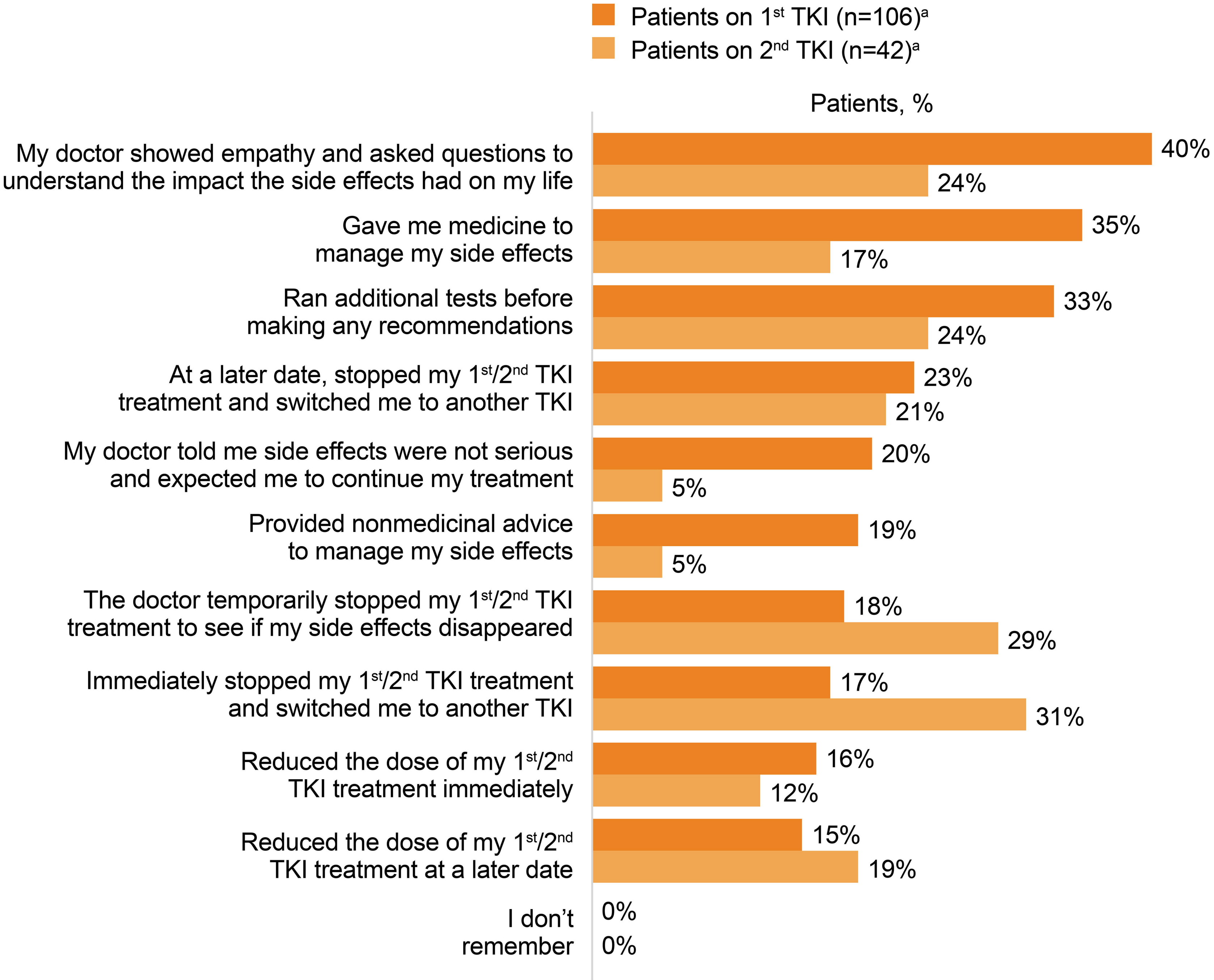
Treatment has no or manageable side effects

Impact on your work life

Impact on mental health

Impact on your financial situation

Impact on your studies or school


Patients on 1st TKI (n=106)a
Patients on 2nd TKI (n=42)a
Patients, %
My doctor showed empathy and asked questions to understand the impact the side effects had on my life
40%
24%
Gave me medicine to manage my side effects
35%
17%
Ran additional tests before making any recommendations
33%
24%
At a later date, stopped my 1st/2nd TKI treatment and switched me to another TKI
23%
21%
My doctor told me side effects were not serious and expected me to continue my treatment
20%
5%
Provided nonmedicinal advice to manage my side effects
19%
5%
The doctor temporarily stopped my 1st/2nd TKI treatment to see if my side effects disappeared
18%
29%
Immediately stopped my 1st/2nd TKI treatment and switched me to another TKI
17%
31%
Reduced the dose of my 1st/2nd TKI treatment immediately
16%
12%
Reduced the dose of my 1st/2nd TKI treatment at a later date
15%
19%
I don't remember
0%
0%

Supplementary Appendix

Supplementary Methods

Qualitative interviews

In the qualitative phase, 60-minute in-depth qualitative interviews were conducted with 21 adult patients who had been treated with ≥1 prior TKI and 24 physicians treating patients with CML in France, Germany, Japan, and the US to identify the appropriate topics for the quantitative surveys.

Quantitative surveys

Quantitative surveys took approximately 40 minutes for patients and approximately 30 minutes for physicians to complete. Questions for both patients and physicians were related to CML diagnosis, treatment, impact on daily life, treatment switching, and shared decision-making. In addition, physicians were asked about disease monitoring.

Analysis

Reported results were derived from survey questions asked as follows:

Information sharing at diagnosis and treatment switching. Patients were asked to select the 5 most important areas of information that they wanted to receive at diagnosis/switch out of 17 options and then ranked in order of importance from 1 to 5, with 1 being the most important. Physicians were asked to select any information they provided to patients at diagnosis/switch (no ranking) out of 16 options.

Treatment goals. Patients were asked to rank their 3 most important goals, and physicians were asked to select any goals.

Shared treatment decision-making. Physicians were asked on a scale from 1 to 7, where 1 means "strongly disagree" and 7 means "strongly agree" to indicate how much they agreed with the following statement: “CML patients' input to a treatment decision is welcomed, but ultimately, I am the decision-maker.” Patients were asked how treatments were described by and agreed upon with their physicians. Physicians were asked how many TKI options they offered and to what extent their patients were involved in treatment decisions.

Burden of disease. Patient perceptions of how CML treatment affects their life were assessed by asking them how much they agreed with certain statements related to treatment impact.

Side effects. Patients were asked to recall conversations with their doctor about side effects, including who initiated the conversations and how the doctor reacted when informed about side effects.

Adherence. Patients were asked to choose reasons for missed doses from a list of 15 options. Physicians were asked what they thought were the reasons for their patients missing doses of their TKI medication from a list of 11 options.

A. Supplemental Tables

Supplemental Table S1. Eligibility criteria

Patient inclusion criteria[a]
≥18 years of age and diagnosed with CML-CP
Not currently employed by a market research company, advertising agency, or a pharmaceutical/biotechnology company
Received 1 to 3 prior TKIs and currently receiving their second, third, or fourth TKI
Physician inclusion criteria[b]
Transplant specialist or hematologist and/or oncologist in practice for 3 to 35 years and personally responsible for treatment decisions for patients with CML-CP
Not currently employed by a market research company, advertising agency, or a pharmaceutical/biotechnology company
Spent ≥50% of their time caring for patients
Switched patients' treatment from their second to third TKI

CML-CP, chronic myeloid leukemia in chronic phase; TKI, tyrosine kinase inhibitor.

[a] Patients were recruited via open internet invitation through patient databases, physician and nurse referrals, and patient advocacy groups. [b] Physicians were recruited via open internet invitation through online physician panels.

Supplemental Table S2. Countries of physician and patient origin

Country	Patient sample/proportion of total, n (%)	Physician sample/proportion of total, n (%)
Australia	20 (6)	13 (7)
Brazil	42 (12)	15 (8)
Canada	10 (3)	15 (8)
France	15 (4)	20 (10)
Germany	37 (10)	20 (10)
Italy	25 (7)	20 (10)
Japan	34 (9)	15 (8)
South Korea	40 (11)	10 (5)
Spain	24 (7)	20 (10)
UK	44 (12)	20 (10)
US	70 (19)	30 (15)
Total	**361/100**[a]	**198/100**[b]

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[a] A total of 3,671 patients clicked on the survey link; 3,310 screened out/did not qualify, while 361 qualified and completed the survey. An additional 626 patients expressed interest in joining the survey but were unable to participate because the respective quotas were already reached.

[b] A total of 327 physicians clicked on the survey link; 129 screened out/did not qualify, while 198 qualified and completed the survey. An additional 111 physicians expressed interest in joining the survey but were unable to participate because the respective quotas were already reached.

Supplemental Table S3. Physician and patient demographics

Patients	n=361	
Female, %	56	
Age, median (range), years	45 (19-82)	
Time from diagnosis of CML, median (range), years	5 (1-33)	
Type of insurance coverage, %		
Public insurance only	33	
Public insurance plus private supplementary insurance	27	
Other[a]	18	
Private insurance only	17	
Public assistance program[b]	5	
Currently receiving treatment by line at the time of the survey, %		
Second TKI	66	
Third TKI	24	
Fourth TKI or later	9	
TKI treatment, %	**Current**	**Past**
Dasatinib	26	36
Nilotinib	16	24
Imatinib	16	45
Generic imatinib	12	17
Bosutinib	10	13
Ponatinib	7	9
Generic dasatinib	6	8
Asciminib	6	4
Radotinib	NR	18
Physicians	**n=198**	
Hematologists, %	41	
Hematologist-oncologists, %	59	
Time in practice, median (range), years	18 (3-31)	
Practice setting, %		
University teaching hospital	59	
Community or general hospital	17	
Cancer center	15	
Office-based (solo or group practice)	8	
General hospital	1	
Time spent on patient care, median (range), %	90 (50-100)	
Patients treated for hematologic cancers over last 12 months, median (range), n	230 (15-3200)	
Patients with CML-CP seen in last 12 months, median (range), n	35 (10-600)	

Patients with CML-CP seen in last 12 months by practice setting, mean (SD), n	
University teaching hospital (n=117)	62 (80)
Community or general hospital (n=34)	84 (135)
Cancer center (n=30)	72 (84)
Office-based (solo or group practice) (n=16)	38 (36)
General hospital (n=1)	15 (0)
Patients with CML on each line of treatment seen in the last 12 months, median (range), %	
First TKI	50 (10-95)
Second TKI	27 (10-95)
Third TKI	20 (0-90)
Fourth TKI or later	9 (0-90)

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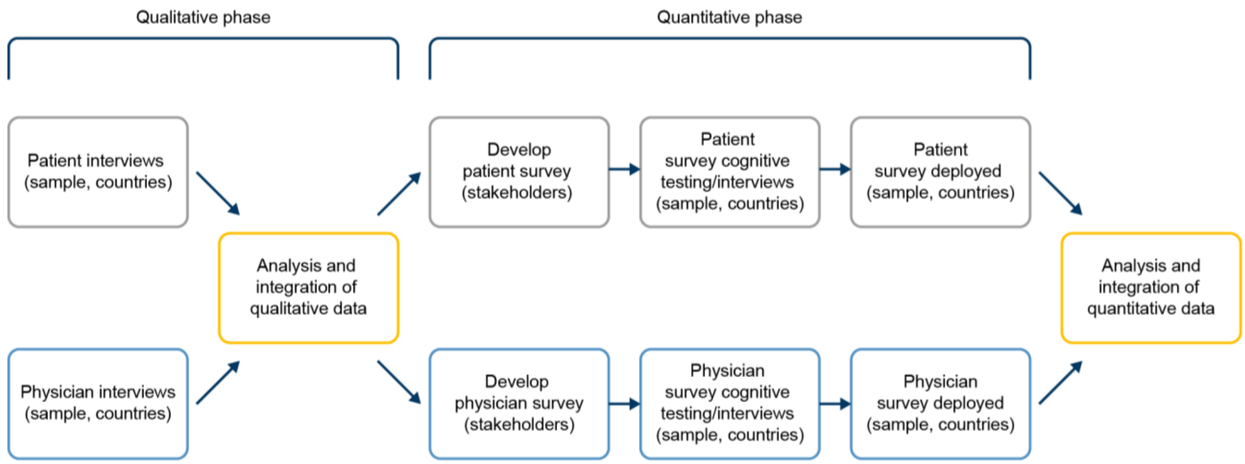
CML, chronic myeloid leukemia; CML-CP, chronic myeloid leukemia in chronic phase; NR, not reported; SD, standard deviation; TKI, tyrosine kinase inhibitor.

[a] Other includes employer-provided, private self-paid, health insurance exchange, military, private insurance plus Medicare, National Health Insurance, and none. [b] Public assistance program includes Medicare, Medicaid, disability, and social security insurance.

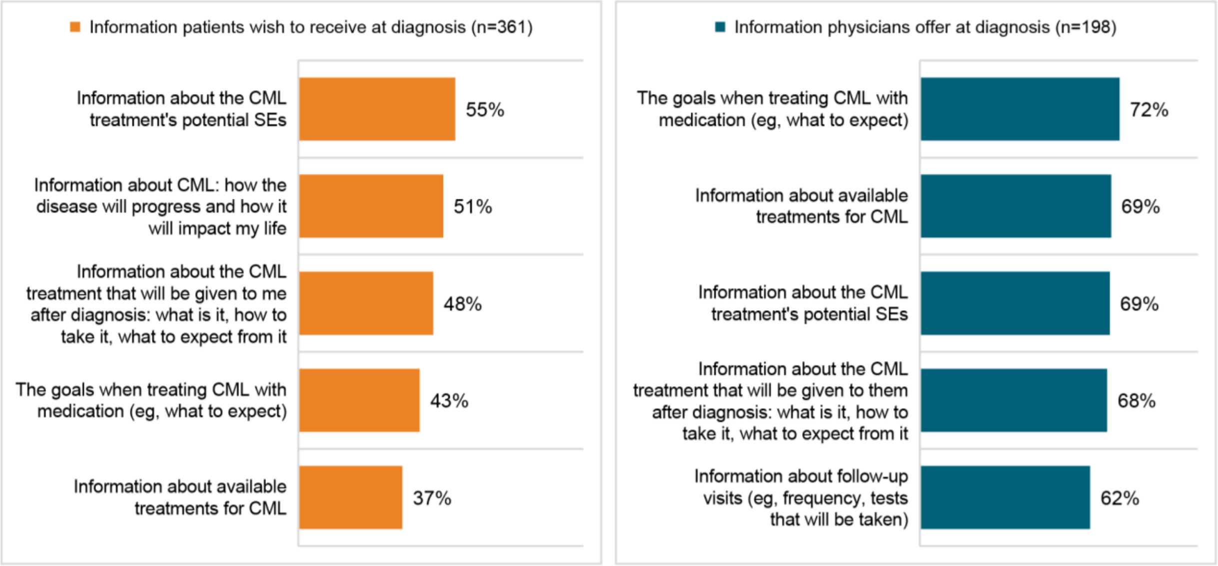
B. Supplemental Figures

Figure S1. CML SUN study design. A mixed methods research with exploratory sequential design. After each phase, qualitative then quantitative, results from both patients and physicians were analyzed and integrated.



CML SUN, Chronic Myeloid Leukemia Survey on Unmet Needs.

Figure S2. Top 5 most important areas of information patients want to receive vs what physicians give at diagnosis. Top 5 areas of information related to CML-CP that patients want to receive at diagnosis ranked in order of preference compared with what physicians actually provide, as assessed by proportions of patients and physicians.



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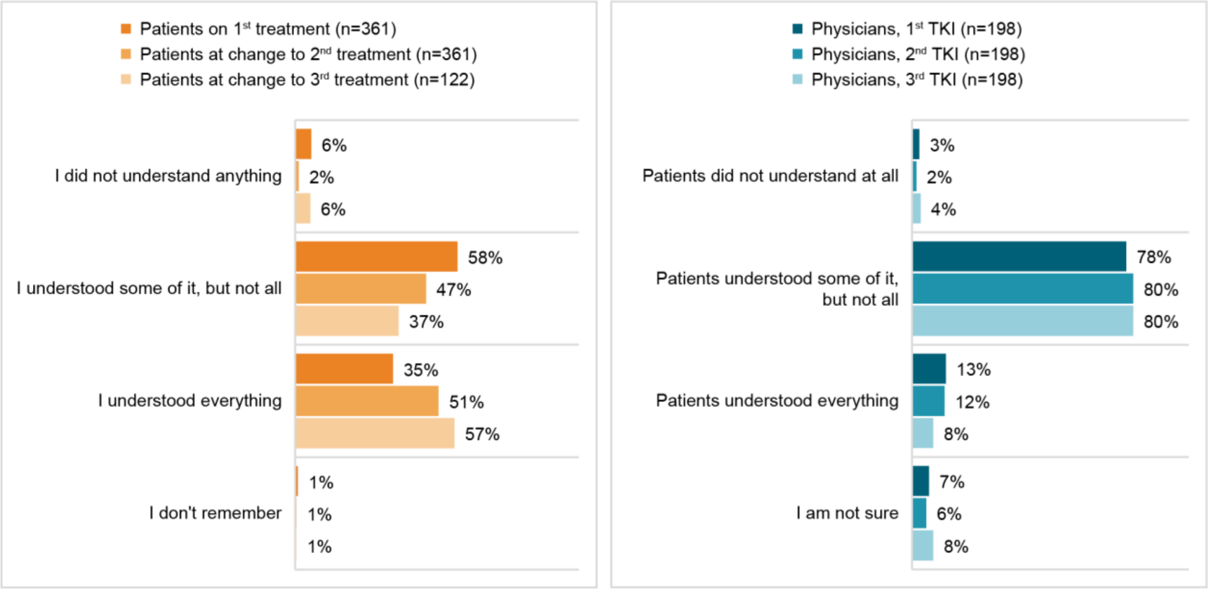
CML, chronic myeloid leukemia; CML-CP chronic myeloid leukemia in chronic phase; SE, side effect.

Figure S3. Patients' understanding of information about treatment. Different levels of patient understanding of treatment information as reported by patients and physicians and assessed by proportions of patients and physicians by sequence of TKIs



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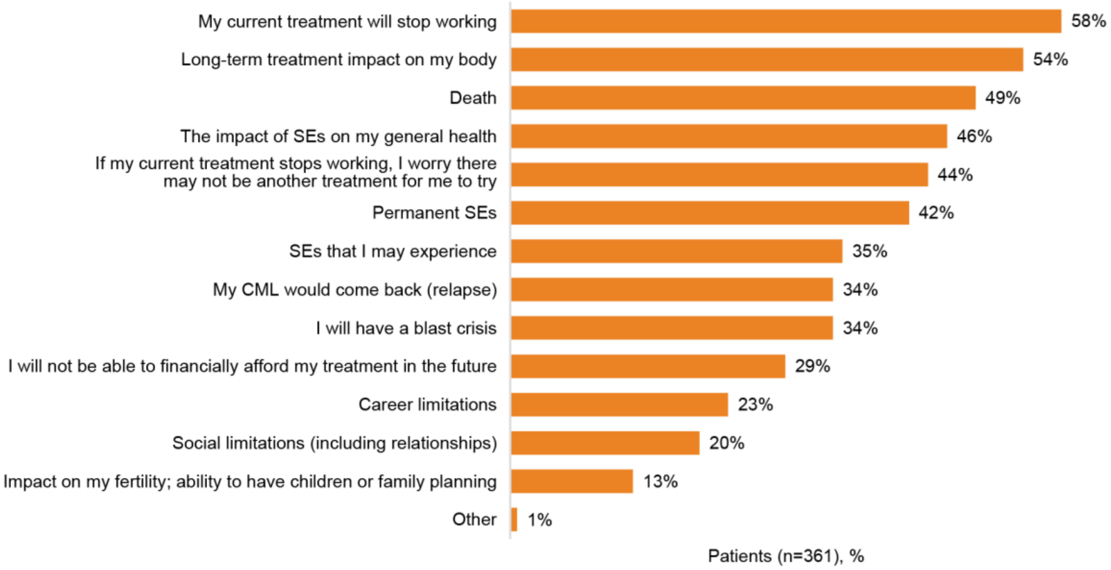
TKI, tyrosine kinase inhibitor.

Figure S4. Patients' fears about CML-CP. Proportions of patients with CML-CP who fear how their disease may impact them.



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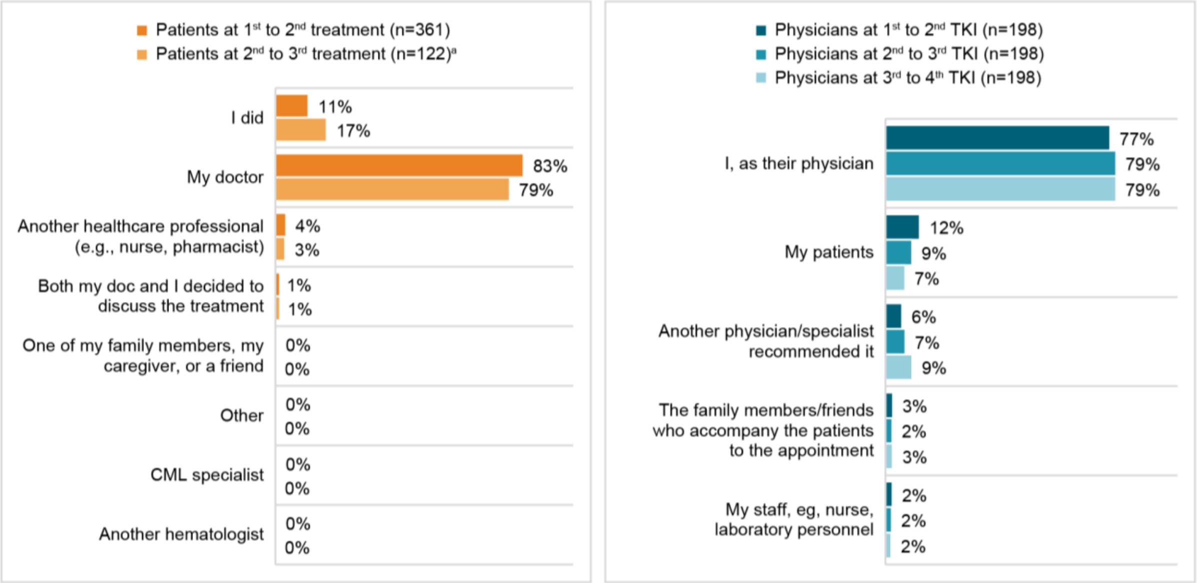
CML, chronic myeloid leukemia; CML-CP, chronic myeloid leukemia in chronic phase; SE, side effect.

Figure S5. Initiation of the discussion on treatment change. Who specifically initiated the discussion on treatment change as reported by patients and physicians, assessed by proportions of patients and physicians by sequence of TKIs.



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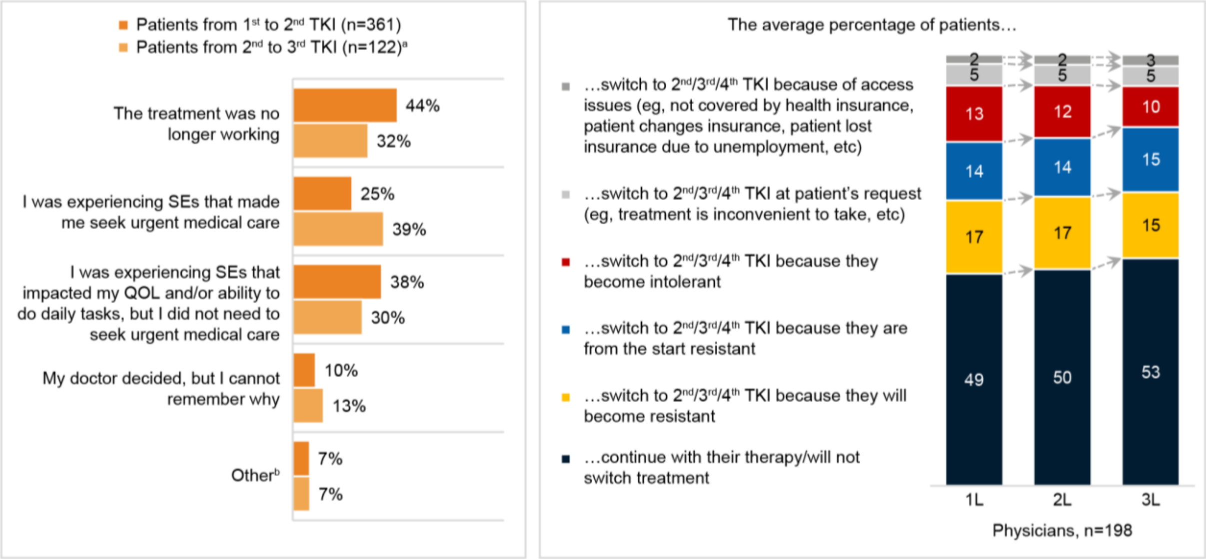
CML, chronic myeloid leukemia; TKI, tyrosine kinase inhibitor.

[a] Included only patients on their third and fourth TKI.

Figure S6. Reasons for switching treatment. Reasons for switching treatment for CML-CP as reported by patients by sequence of TKIs and physicians, assessed by proportions of patients.



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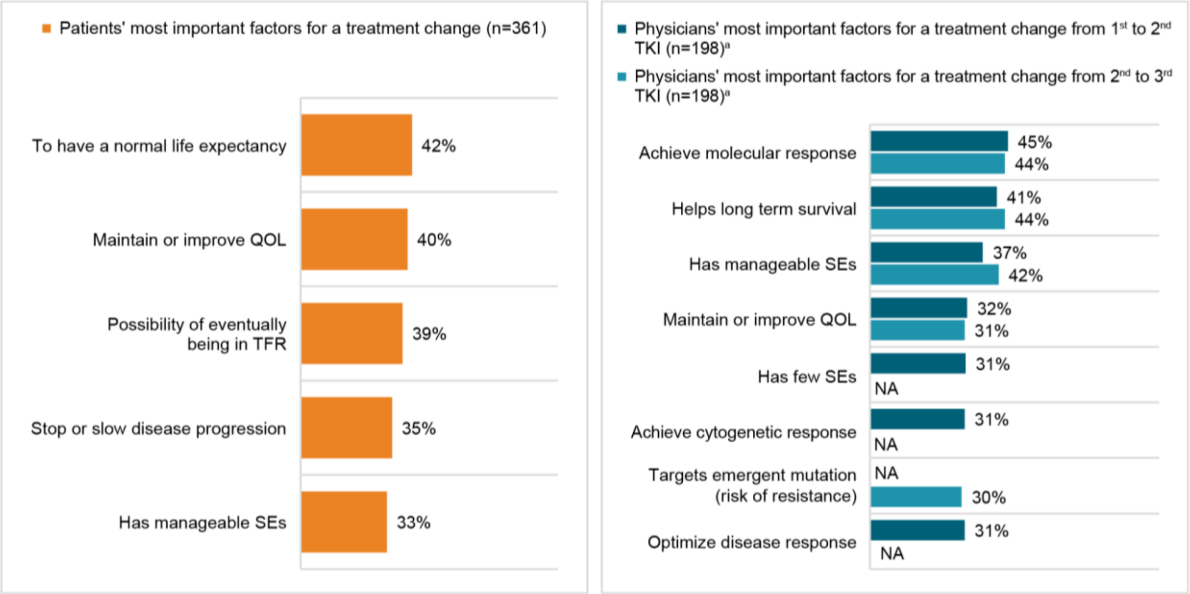
CML-CP, chronic myeloid leukemia in chronic phase; QOL, quality of life; SE, side effect; TKI, tyrosine kinase inhibitor.

[a] Included only patients on their third and fourth TKI. [b] "Other" is a sum of responses to: other, recommended by the doctor, better availability/accessibility, for faster onset of action, increased frequency of administration, poor adherence to treatment, restriction on meal time, based on the guidelines/protocol, I participated in the clinical trial, I wanted to conceive/get pregnant, new treatment option, comorbidities, received funding for the treatment, none, don't know, and refused/no answer.

Figure S7. Top 5 most important factors for patients and physicians when considering a treatment change. Five most important factors to physicians when considering a treatment change, by sequence of TKIs, compared with the 5 most important factors to patients when considering a treatment change, assessed by proportions of physicians and patients.



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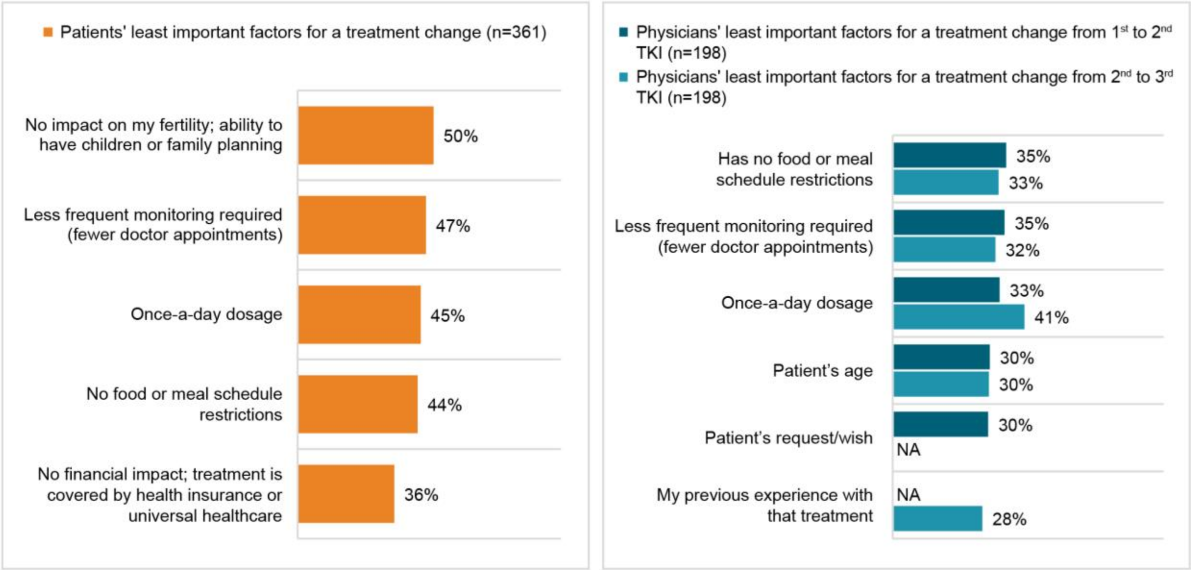
NA, no answer; QOL, quality of life; SE, side effect; TFR, treatment-free remission; TKI, tyrosine kinase inhibitor.

[a] Besides enabling patients to live longer.

Figure S8. Top 5 least important factors for patients and physicians when considering a treatment change. Five least important factors to physicians when considering a treatment change, by sequence of TKIs, compared with the 5 least important factors to patients when considering a treatment change, assessed by proportions of physicians and patients.



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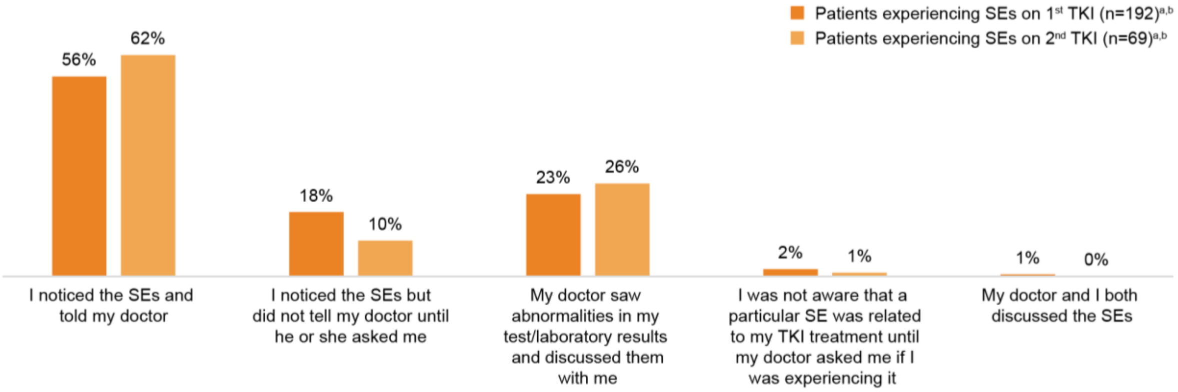
NA, no answer; TKI, tyrosine kinase inhibitor.

Figure S9. Roles in initiating side effect discussions. Different ways that discussions around side effects were initiated as assessed by proportions of patients on first and second TKI therapy.



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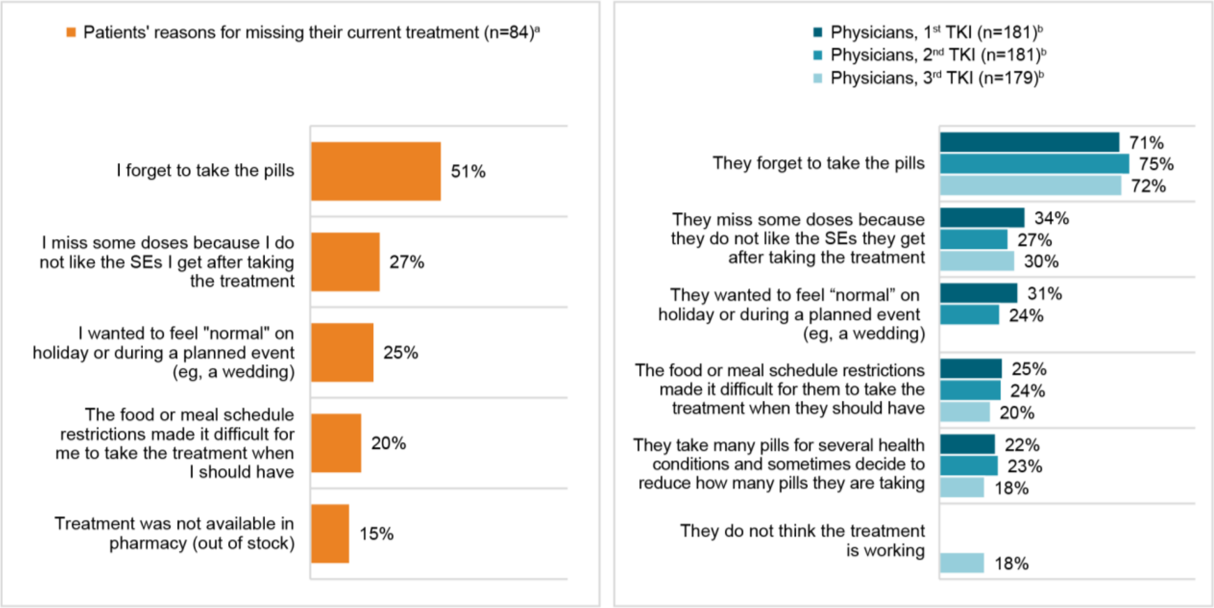
SE, side effect; TKI, tyrosine kinase inhibitor.

[a] Included patients who responded that they switched treatment because of side effects. [b] No patients selected “other”.

Figure S10. Top 5 reasons for missed doses. Top 5 reasons for missed doses from the physician perspective, as assessed by the proportion of physicians by number of TKIs and from the patient perspective, as assessed by the proportion of patients.



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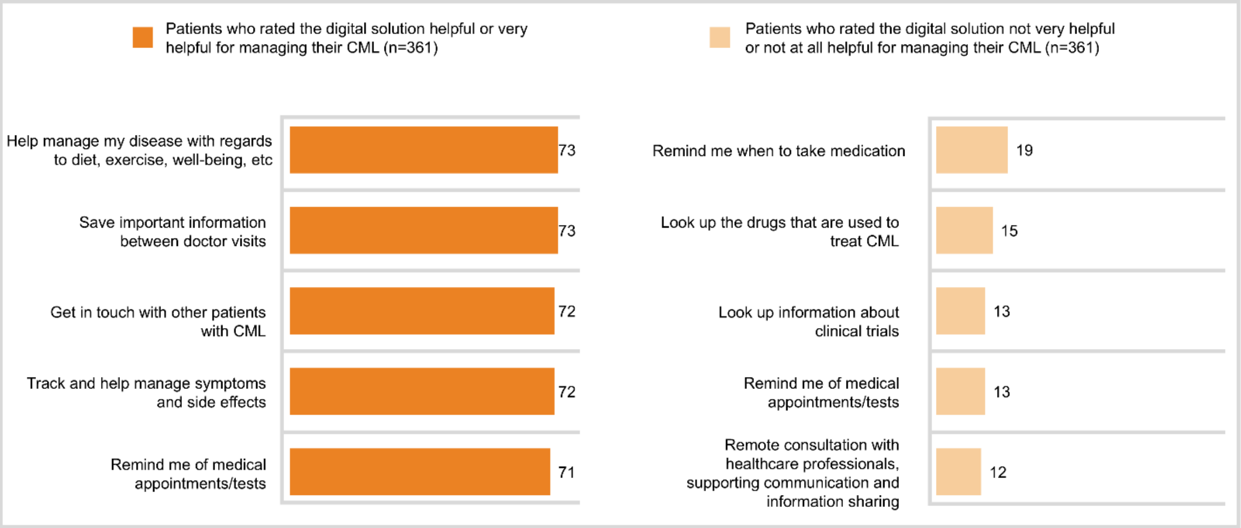
SE, side effect; TKI, tyrosine kinase inhibitor.

[a] Included patients who reported being nonadherent ≥1 time. [b] Included physicians who thought patients may be nonadherent.

Figure S11. Top 5 and bottom 5 digital healthcare solutions for managing CML (patient perspective).



CML, chronic myeloid leukemia.