



Survey Results: Oral Vancomycin and Insurance Coverage

PSC Partners received a direct request from patients to survey the PSC patient community on their experience with insurance coverage of their medication, particularly oral vancomycin¹, in advance of Rare Disease Day, February 28, 2023. This data will be used in part to inform legislative advocacy in support of H.R. 6160, Access to Rare Indications Act of 2021².

A short, IRB-approved survey was administered through the PSC Partners Patient Registry.³ The survey was open for 5 days in February 2023 (February 22 - February 26). Invitations to complete the survey were posted on the PSC Partners' two Facebook support groups and other social media, emailed directly to patients and caregivers from the PSC Partners mailing list, and emailed from the Registry directly to those Registry participants who had previously indicated they had ever taken oral vancomycin for PSC. The outreach and response to the survey is summarized in Table 1.

Table 1: Summary of Survey Outreach and Response		
Outreach and Response	<i>n</i>	Notes
Approximate number of individuals (patients and caregivers) who received outreach messages via direct emails to general mailing list, direct emails to Registry participants, and posts to Facebook support groups/other social media (twitter, instagram, LinkedIn) ⁴	6000	Sent Feb 22, 2023
Approximate number of Registry participants who had previously indicated they had ever taken oral vancomycin for PSC	300	E-mailed and re-sent to those who did not open
Total Number of responses	79	
Total Number respondents who responded YES to the question if they have EVER taken OV for PSC	73	5 responded no 1 responded not sure (in clinical trial)
Number of days survey open	5	

¹ [PSC Partners Statement about the Use of Medications Not Approved by the FDA for PSC](#). A listing of peer-reviewed publications related to oral vancomycin for PSC can be found on the PSC Partners Patient Registry page titled [Publications Related to Clinical Trials in PSC](#) and located under the heading *Mayo Clinic: Oral Vancomycin for PSC*.

² Information can be found at <https://www.congress.gov/bill/116th-congress/house-bill/6160>

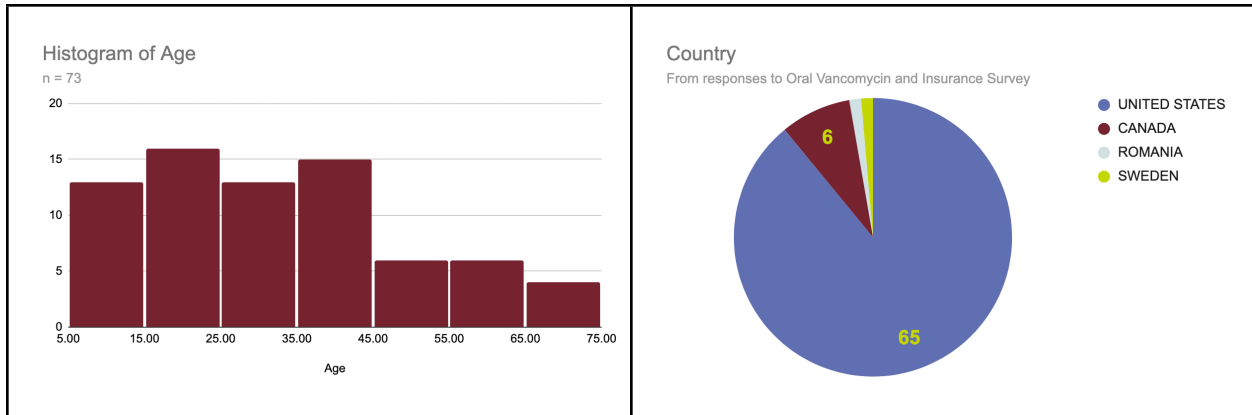
³ www.pscpartnersregistry.org The PSC Partners Patient Registry was established in collaboration with the Office of Rare Diseases Research (ORDR), National Center for Advancing Translational Sciences (NCATS), the National Institutes of Health.

⁴ Estimated number of individuals in the US living with a diagnosis of PSC is 30,000-50,000.

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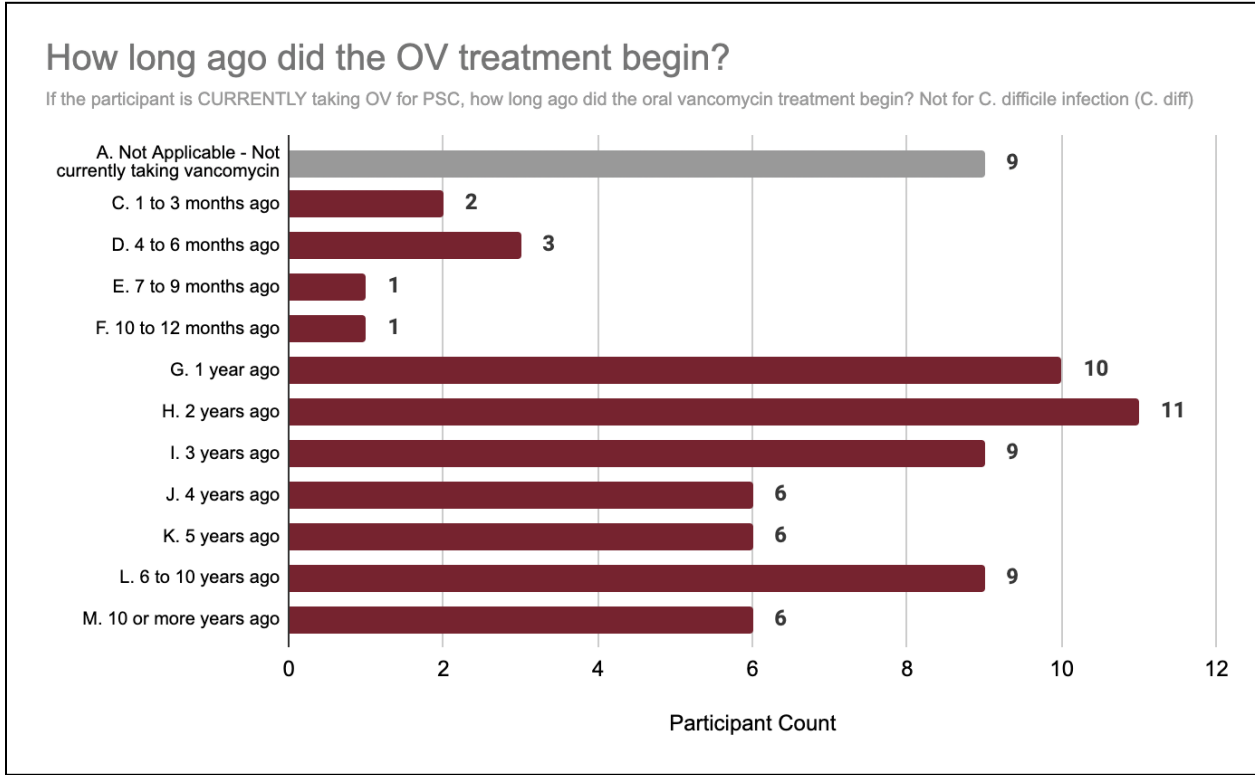
Table 2 presents characteristics of the 73 respondents who completed this survey and have EVER taken OV for PSC.

Table 2: Characteristics of Survey Respondents (n=73)		
Features	Data	Notes
Range of age	7-73	
% aged 25 years or less	39.7%	Many US-based pediatric patients have access to pediatric care until age 25 or younger.
Reported gender	Female 39.7% Male 60.3%	
Number of countries represented ⁵	4	US (65), Canada (6), Romania (1), Sweden (1)
Number currently taking OV	64	
Number who have taken OV in the past, not currently taking	9	
Measure of how long participant has been taking OV (if currently taking)	Ranges from 1 month to more than 10 years. See <i>Histogram of Age</i> chart.	Survey Question: <i>If the participant is CURRENTLY taking oral vancomycin (Vancocin) for PSC, how long ago did the oral vancomycin treatment begin? Not for C. difficile infection (C. diff)</i>

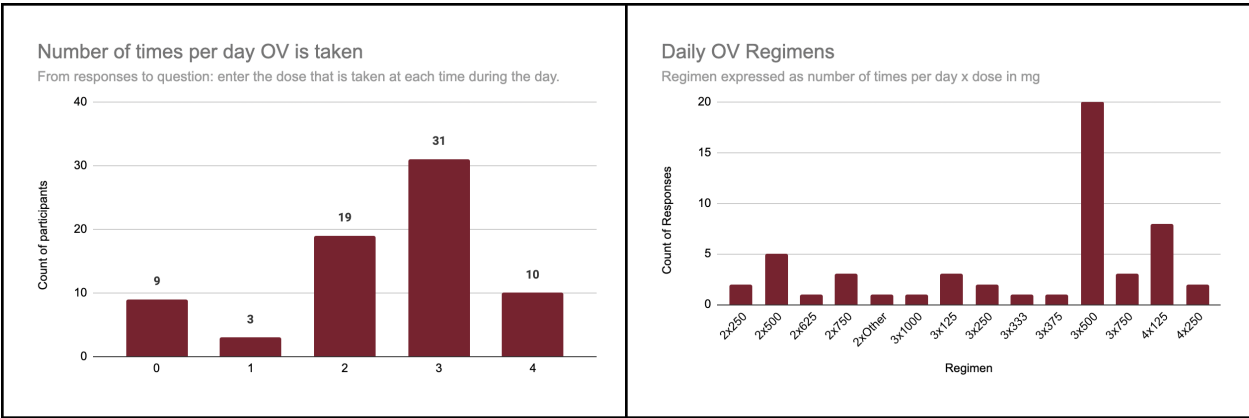


⁵ Country data as reported by Registry participants in their Registry account. This information is collected when a participant joins the Registry, and can be updated at any time. The OV and Insurance Coverage survey collected the number of respondents living outside of the US with the question "What state do you live in? (Other, if outside of US)?" The number of Other responses in the survey matches the number of respondents with a non-US country in their Registry account.

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Oral Vancomycin Regimens: The reported daily regimen for OV use ranged from once a day to four times a day. Doses ranged from 125 mg to 1000 mg at a time. Daily total use ranged from 125 mg to 3000 mg. The most common regimen (n=20) reported was 500mg three times per day.

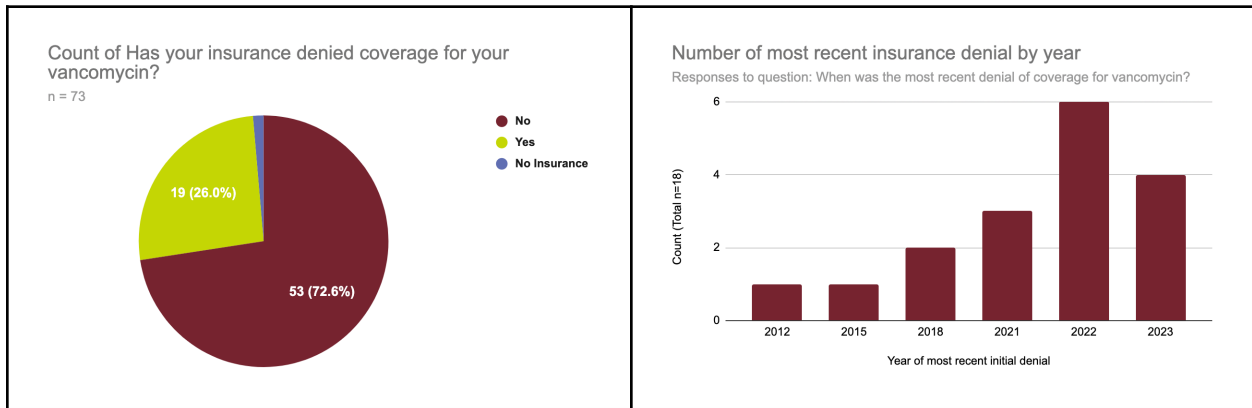


n=72 in these regimen charts. One respondent did not submit dose regimen information.

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Insurance Coverage, Denials and Appeals: The survey next asked about insurance coverage for oral vancomycin for PSC with the following question: *“Has your insurance denied coverage for your vancomycin?”* A majority (72.6%, n=53) of those responding have not had an insurance denial for OV. However, some (26.0%, n=19) report experiencing an insurance denial. Please see Addendum 1 for responses to survey question *“What reason did the insurance provide for denying vancomycin (Vancocin) coverage?”*

Of the 19 experiencing a denial, 11 reported submitting an appeal of the denial, 5 reported planning to submit an appeal, and 3 did not submit an appeal. After appeal, 7 of the 11 reported that their appeals resulted in an approval for insurance coverage of OV for PSC⁶. 1 respondent of the 11 responded “Other” as the outcome of the appeal⁷ but indicated that they have found a way to obtain repeated short-term approvals that they are piecing together. And the remaining 3 reported that insurance denied the appeal. In summary, 3 of 73 respondents were denied insurance coverage⁸ upon appeal for OV.



Of the 3 with insurance denial following appeal, the insurance provider for 2 is Medicare, and the third did not indicate their insurance provider. One respondent with a standing denial of insurance coverage for OV says “I had no idea how to file an appeal; I was unaware that I had only two times to appeal the denial. Vancomycin has improved my quality of life. I have had no adverse effects. Labs normalized after 3 months of starting this dose and Crohn’s has been controlled.” Another respondent said “My vancomycin was covered by Oscar Insurance, Aetna, and United Healthcare previously, but not by Medicare.”

Of those who received an initial denial of coverage for OV from their insurance, 8 did not submit an appeal. 3 of these respondents plan to submit an appeal. It appears from their comments that these three may have recently switched insurance providers. The remaining 5 respondents answered No to the question *“Have you submitted an appeal of the denial?”* 3 of these 5 are

⁶ Of the seven approvals upon appeal, Blue Cross Blue Shield, United Healthcare were named as having approved OV upon appeal. (Some respondents indicated their insurance was not listed in the survey.)

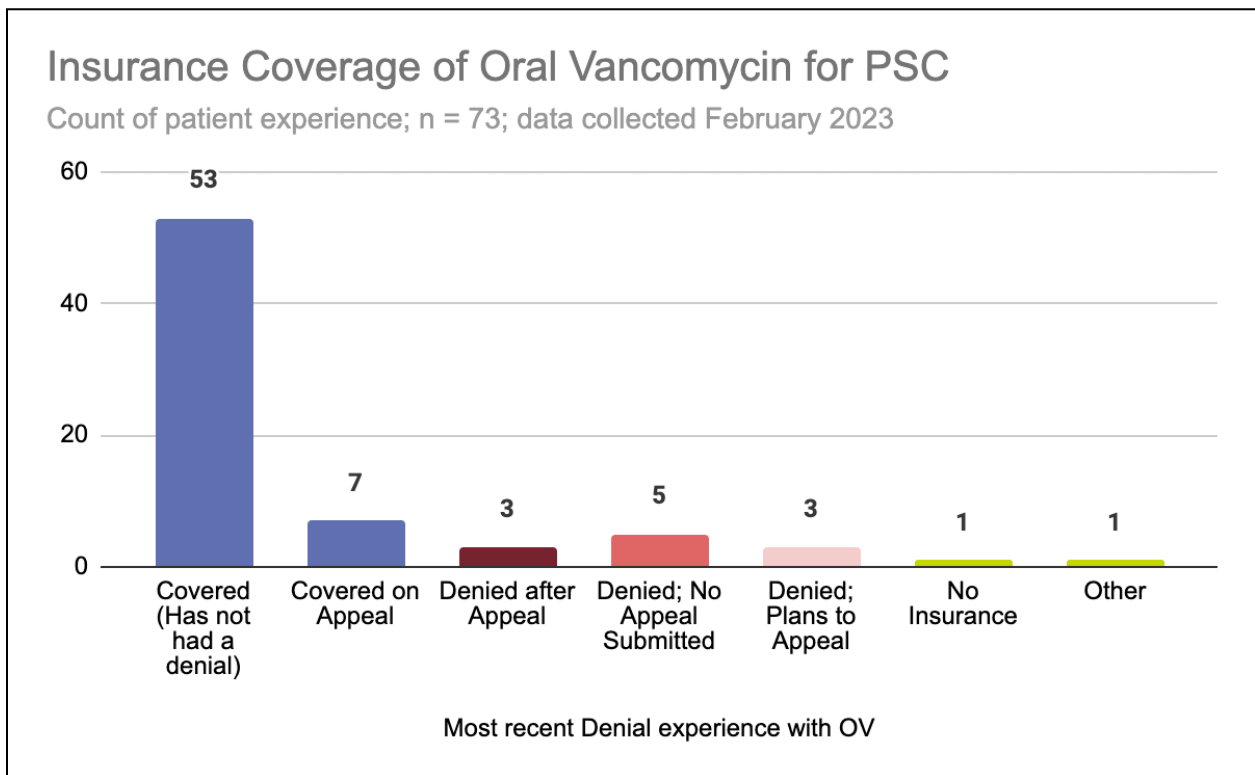
⁷ In this one case, the insurance company denied the appeal, and the response indicates that the pharmacist has been able to work around this denial by submitting authorization requests every 10 days, with which the individual has been able to receive insurance coverage. *“Pharmacist split the prescription into 10 day duration that did not get denied. Then I would refill it every 10 days.”*

⁸ Note: Of the 3 documented denials, 2 were prior to 2022, the year of the newly released AASLD Practice Guidance on PSC and CCA.

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not currently taking OV, although one indicates in comments that “I would love to have it covered as the out of pocket cost is not cheap.” This raises the question of what the reason is that this respondent is not currently taking OV. Of the 2 remaining of these 5, one appears to have moved to the Cost+ pharmacy to manage while the other is in Canada with a different insurance system.

The following chart summarizes the insurance coverage of OV for PSC as represented by the data collected in this survey. The survey did not ask any questions specifically relating to the burden of managing this process. For additional open-ended feedback on oral vancomycin and insurance coverage, please see Addendum 2 for comments⁹ submitted in response to “*Any other information/comments you'd like to share about oral vancomycin and insurance coverage?*”



The PSC Partners Registry team thanks the many community members who have joined the Registry and who respond to calls for participation.

We are together in the fight, whatever it takes.

⁹ Edited to remove identifying information and information outside the scope of advocacy for H.R. 6160.

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Addendum 1: Responses to survey question “What reason did the insurance provide for denying vancomycin (Vancocin) coverage?”

- Only approved for short-term use so after the first 3 months, they stopped covering it
- Appropriateness of medication
- Vanco not a treatment for PSC
- Would not give a month’s supply. Need to pay every two weeks. When deductibles are met, we need to have the doctors fill out forms as to why the patient needs it
- Not fda approved therapy
- They said it wasn’t listed as a treatment for PSC
- They did not understand that it needed to be taken continuously. They gave short-term approvals and sometimes required next-level appeals to once again authorize it.
- Not approved by government
- Not an approved dosage, not an approved use. Appealed twice, no approval..

Addendum 2: Selected comments¹⁰ submitted in response to “Any other information/comments you’d like to share about oral vancomycin and insurance coverage?”

- It’s expensive. Over \$1000 for 2 weeks.
- My vancomycin is approved three months at a time. Every three months my doctor has to submit a request for coverage in writing. So far approval has been extended each time. My doctor warns me there will come a day when this will not be the case. It’s incredibly deflating.
- I can’t believe we are being denied after 10 years of use, hopefully guidelines will be corrected.
- At first, I took Prasco brand, then ANI. Then a prior insurance stopped covering that brand. By then it was obvious that it wasn’t helping my PSC, but it was helping my UC. I gave the non-preferred generic a trial and it kept working, so I never appealed for the preferred generic.
- My Hepatologist appealed three times! They didn’t care!
- Switched to Mark Cuban Cost plus Drugs which sells Lupin Vancomycin at cost plus a mark up.
- I would love to have it covered as the out of pocket cost is not cheap
- Anthem blue cross did not cover the vanco in 2022. We paid upwards of 15k out of pocket to keep him on vanco.
- My vancomycin was covered by Oscar Insurance, Aetna, and United Healthcare previously, but not by Medicare. I currently use a coupon to make it cheaper. It was “covered” at points costing \$200-\$250 per month.
- Canadian, so insurance covered Rx for Vanco without issue as it was prescribed by hepatologist.
- I did not experience any clinical or personal benefit to oral vancomycin treatment for psc despite years of being on it. At the time of taking it I was covered by Tricare insurance and lived in Alaska, but the time period was more than 10 years ago.

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- *I believe required a PA with initial start but never denied since starting Feb 2021.*
- *We are only approved for drug on a 3 month basis and physician has to resubmit again for continuation of the drug; so far no denial just a process. Drug costs us \$551 monthly and presume once we hit our high deductible our cost would go down to \$120.*
- *I have been lucky enough to only ever have a max OOP cost of \$25 for a 3 month supply.*
- *Insurance in Ontario Canada has covered it without question or appeal x 1 year.*
- *It has been working great!*
- *Very concerned about maintaining my coverage for this unjustifiably overpriced medication that works, has me in remission; I do not wish the risk of rPSC deterioration or another transplant.*
- *When I first started Vanco, it was denied by insurance. I had to fight for it because I exhausted all other medication options. Every medication for PSC and UC went toxic in my system.*
- *I have heard of many patients getting denied already.*
- *Patient's PSC has been more or less quiescent, but oral vanco has increased energy and cleared up the ongoing diarrhea that former GI doctor insisted wasn't a concern.*
- *I am currently still allowed to pick up my Rx from a local pharmacy. This is critically important because oral vancomycin is manufacturer specific when used to treat PSC. I use vancomycin made by Ani Pharmaceuticals. Mail order pharmacies cannot guarantee any specific manufacturer for a generic medication. If I am ever forced to use mail order for my oral vancomycin, it may not be nearly as effective at keeping my PSC and UC in remission, due to switching manufacturers. It's very important for patients to always have a choice in pharmacies.*
- *I do not have coverage until I surpass my deductible and then 100% coverage once I have passed my Out of Pocket Max. My deductible is \$8550 and my annual max is \$14K. The cost before coverage is anywhere between \$400-\$600 for a monthly supply. My provider is Regence BC BS (specifically the Participating Network which is their most accessible network option).*
- *It had made a huge difference in my health and has likely saved the insurance company a lot of money.*
- *I've tried tapering down on the vancomycin and that caused the inflammation in my gut to increase dramatically.*
- *Oral vancomycin has literally saved my young patient's life. Diagnosed with Primary Sclerosing Cholangitis (PSC) and Ulcerative Colitis early at age five. Liver biopsy showed Stage 3 fibrosis. Extremely sick – enlarged spleen, ascites, no appetite, white stool, loose stool, losing weight, fatigue, etc. We immediately began researching the disease and came across Dr. Kenneth Cox's research at Stanford in which he successfully treated children with PSC using oral vancomycin. We located a liver specialist who was willing to prescribe vancomycin for PSC, as he has been treating his own patients successfully for years. Patient's liver numbers before starting oral vancomycin were off the charts.*
- *OV is an extremely expensive co-pay, but to date, has not been denied.*

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- *Though my insurance has (thankfully) not denied coverage at this point in time, I do have to submit an annual Pharmaceutical Exception Request to get approval for the number of capsules I require each month. It's a lengthy and stressful process I go through every year, but I'm so grateful I have access to this medication. There's always a looming fear that I could lose coverage at any moment. I hope and pray I will not lose coverage for this medication that has given me my life back.*
- *I have had it temporarily denied or limited in quantity or weird authorizations required. It also took longer and was harder to get it back in 2012 before the patent expired and generics became available. Since then it has been actually less hassle than expected. Getting the authorized generic ANI which works the best. I have heard some horror stories from various patients who had a hard time getting through the bureaucracy, or had to use liquid vancomycin blended into a syrup. I have experienced a certain amount of pointless, bureaucratic infighting, but ultimately was always able to get the medication at the end of the day.*
- *Oral Vancomycin was initially prescribed because of research I found online and a prior stint of C-Diff. Oral Vancomycin treatment put me in remission from UC for half a year. Symptoms did eventually return. Still taking due to potential evidence of support of both UC/PSC.*
- *Vancomycin has been a game changer for my PSC. Since I have been on it, my labs have been normal.*
- *Saved my life without it I would not be able to graduate from college.*
- *To date, insurance has covered my oral vanco. I have not had a bout of cholangitis since I started this medication. I'm very worried if they do start to deny my medication that my disease would progress.*