



## International Rare Cancers Initiative (IRCI) Desmoplastic Small Round Cell Tumour ASCO 2016 Minutes DRAFT

Saturday 4<sup>th</sup> June, 12.00-13.00 (CDT)  
Salon A4, Hilton Chicago Hotel, 720 South Michigan Avenue Chicago, Illinois 60605

**Chair** – Prof. Jeremy S. Whelan

**Attendees:** Alexander Aledo (AA), Danielle Battle (DB, IRCI Secretary), Paolo Casali (PC), Winette van der Graff (WG), Alessandro Gronchi (AG), Doug Hawkins (DH), Pooja Hingorani (PH), Bernd Kasper (BK), Saskia Litiere (SL), Juame Mora (JM), Anastassia Negrouk (AN), Silvia Stacchiotti (SS), Paolo Dei Tos (PDT), Jack Welch (JW), Jeremy S. Whelan (JSW, Chair)

### Agenda item

#### Welcome and approval of IRCI DSRCT Minutes from CTOS Berlin October 2014 (Appendix 1).

JSW welcomed the group and the minutes of the previous meeting held at CTOS were approved.

#### Refreshing group activity and goals

JSW highlighted the proposals for a prospective study and international registry that were previously developed by the DSRCT group (Appendices 2 and 3), and outlined that the purpose of the meeting was to revisit these projects, review previous actions, assess group interest and think about the best ways in which the group can move forward.

#### Previous actions update

JM informed the group that after 2 years of negotiations, Celgene had agreed to sponsor the nab-paclitaxel study trial. The trial is run by the Spanish Sarcoma group and is currently enrolling patients. JM updated that he had provided Celgene with a list contacts in order to promote international participation of the study (expanding the DSRCT cohort). JM invited group members to consider whether there would be any interest from additional EU groups participating in the trial, and suggested that this could be part of a wider international collaborative effort.

**ACTION:** Group members to assess interest and consider participating in the nab-paclitaxel study and feedback to JM.

The additional actions from the previous minutes (as per the agenda) were yet to be completed.

#### International registry developments

PH updated the group with regards to the international registry concept that was previously developed, following a meeting of the DSRCT group in 2013 (Appendix 3). The primary aim of the proposal would be to develop and utilise an international registry that would collect demographic, clinical and outcome data from patients with DSRCT, with the overall objective of advancing the scientific knowledge base of this rare cancer and inform critical outstanding questions in the field. Secondary aims include establishing a repository of patient samples that can be



used for pathological review and scientific research, and developing some standard treatment recommendations for chemotherapy, surgical intervention and other treatment modalities.

PH reiterated that the development of an international registry would be a good strategy in terms of sustaining group momentum and making progress in this disease area. However, since the concept was first proposed at CTOS in 2014, members acknowledged that little progress had been made. Various reasons were attributed to the lack of progress. Firstly, it was considered that the group did not yet have a good sense of how to approach this from a funding standpoint. Members highlighted that there are limited funding opportunities for the development of a rare cancer registry, and obtaining funding through conventional means (e.g. research council/funding body) would be difficult. It was also noted that a registry would need significant data management and cleansing, for which additional funding would need to be sought. JSW asked group members to consider alternative funding mechanisms and potential fundraising/philanthropic routes, and asked how the group might reach out to the DSRCT community. It was suggested that ultra-rare registries may only survive if a family were to provide direct support or the registry is associated with a clinical trial. DB reminded members that there is an IRCI website with a dedicated DSRCT page, and therefore this presented an opportunity to raise the visibility of the group's effort and activities, which may in turn lead to new funding opportunities. However, the information on the DSRCT webpage is currently limited and as such would benefit from some fresh content. DB agreed to help develop some new web content for the group.

**ACTION:** DB to liaise with JSW regarding developing new content for the DSRCT webpage on the IRCI website.

In addition to the difficulties obtaining funding, members also expressed a difference of opinion with regards to what the primary objectives and potential endpoints of the registry should be. Some members also questioned the value and utility of a DSRCT registry, adding that the concept may not work unless there is something more 'attractive' from a research perspective, such as associated translational work. Other members argued that initiating a registry for a rare cancer (such as DSRCT) could be very valuable. The importance of navigating through the issues, establishing a group consensus and agreeing on clearly defined objectives was emphasised.

It was highlighted that following the CTOS meeting in Berlin, the group agreed to start with data collection. It was noted that data collection should, in theory, be straightforward. However, potential regulatory issues were raised as a concern. Other members expressed the view that having a reliable prospective clinical database (which included the collection of tumour tissue) could be worthwhile, and data collected could be used as an external control for future clinical studies. The importance of needing baselines and comparators was discussed. PH suggested that two separate, but comparable registries could be developed (in parallel). One approach could be to pool local retrospective data collections and concentrate efforts in either Europe or the US, until the registry is up and running. The group could then look at expanding the registry internationally. Members agreed that a multi-institutional protocol for collection of retrospective data would be required. JW added that a number of IRCI groups (including Rare Melanoma) are considering developing a rare cancer registry, and it may be worth considering participation in cross-IRCI registry committee.

JSW summarised that the development of a registry would need an enthusiastic champion, full support and commitment from the group, and sufficient funding (either philanthropic or other means). The lack of viable funding options was again raised as a concern, and not all group members agreed the current registry proposal (as

it stands) would add value to the DSRCT community.

**ACTION:** JW to raise at the concept of registries at the IRCI Cross Leads Meeting (ASCO 2016), and consider establishing a cross-IRCI registry committee and feedback to the DSRCT group.

### *Prospective study update*

JSW highlighted the prospective study discussion document that was previously circulated (Appendix 2), and asked members to consider whether there is still an appetite to run a prospective interventional study in DSRCT, and reflect on why little progress has been made. Whilst there was some enthusiasm for developing a prospective study, limited funding opportunities and time resource were considered to be factors hindering progress. It was also proposed that a novel idea or agent was needed in order to motivate researchers and enable this to move forward.

One of the main difficulties is that there is not much known about treatment efficacy and outcome in this rare cancer – and both are essential for the statistical design of a study and any prospective evaluation. SL suggested that in order to work on the study design, more comprehensive information was needed, and a consensus regarding concept feasibility should be reached. Whilst some members agreed that a drug was needed to drive any prospective study forward, there were strong differences of opinion regarding the agent of choice should (e.g. pazopanib). Drug accessibility was also considered to be an issue, as drug availability varies significantly between countries.

The group also discussed the potential questions around surgery and radiotherapy, however, the endpoints would need to be clearly defined. JM suggested that it would be beneficial to test the hypotheses of work that other groups are producing, and agree on an approach and study outputs.

Given the rarity of the disease, the idea of a DSRCT registry was raised again. JSW re-emphasised the importance of being clear about the questions the group would want to have answered from a registry.

### **Recent relevant publications (Appendix 4)**

This item was not discussed in the interests of time but the publications were noted.

### **Summary**

The Chair summarised the discussions from the session and thanked everyone for their contribution. JSW reiterated that there remain several outstanding questions and challenges remain about how best to proceed as a group, and innovative solution was required. AA emphasised that members need to sort out the differences of opinion regarding the registry and prospective study concepts. One solution would be to combine evidence, produce a document and build on that. JSW advised that he would feedback to the IRCI Cross Leads meeting (ASCO 2016) and float the idea of the registry concept with the IRCI Board. JSW also suggested he would solicit some views outside of the group and feedback. The Chair invited the group to consider whether they wish to meet at CTOS Annual Meeting in Lisbon, Portugal, 9-12<sup>th</sup> November.



**ACTION:** JSW to raise registry concept to IRCI Board members at the Cross Leads Meeting (ASCO 2016) and feedback to group

**ACTION:** DSRCT Group to decide date and venue of next meeting

#### Summary of Actions:

1. **ACTION:** Group members to assess interest and consider participating in the nab-paclitaxel study and feedback to JM.
2. **ACTION:** DB to liaise with JSW regarding developing new content for the DSRCT webpage on the IRCI website.
3. **ACTION:** JW to raise at the concept of registries at the IRCI Cross Leads Meeting (ASCO 2016), and consider establishing a cross-IRCI registry committee and feedback to the DSRCT group.
4. **ACTION:** JSW to raise registry concept to IRCI Board members at the Cross Leads Meeting (ASCO 2016) and feedback to group.
5. **ACTION:** DSRCT Group to decide date and venue of next meeting.

